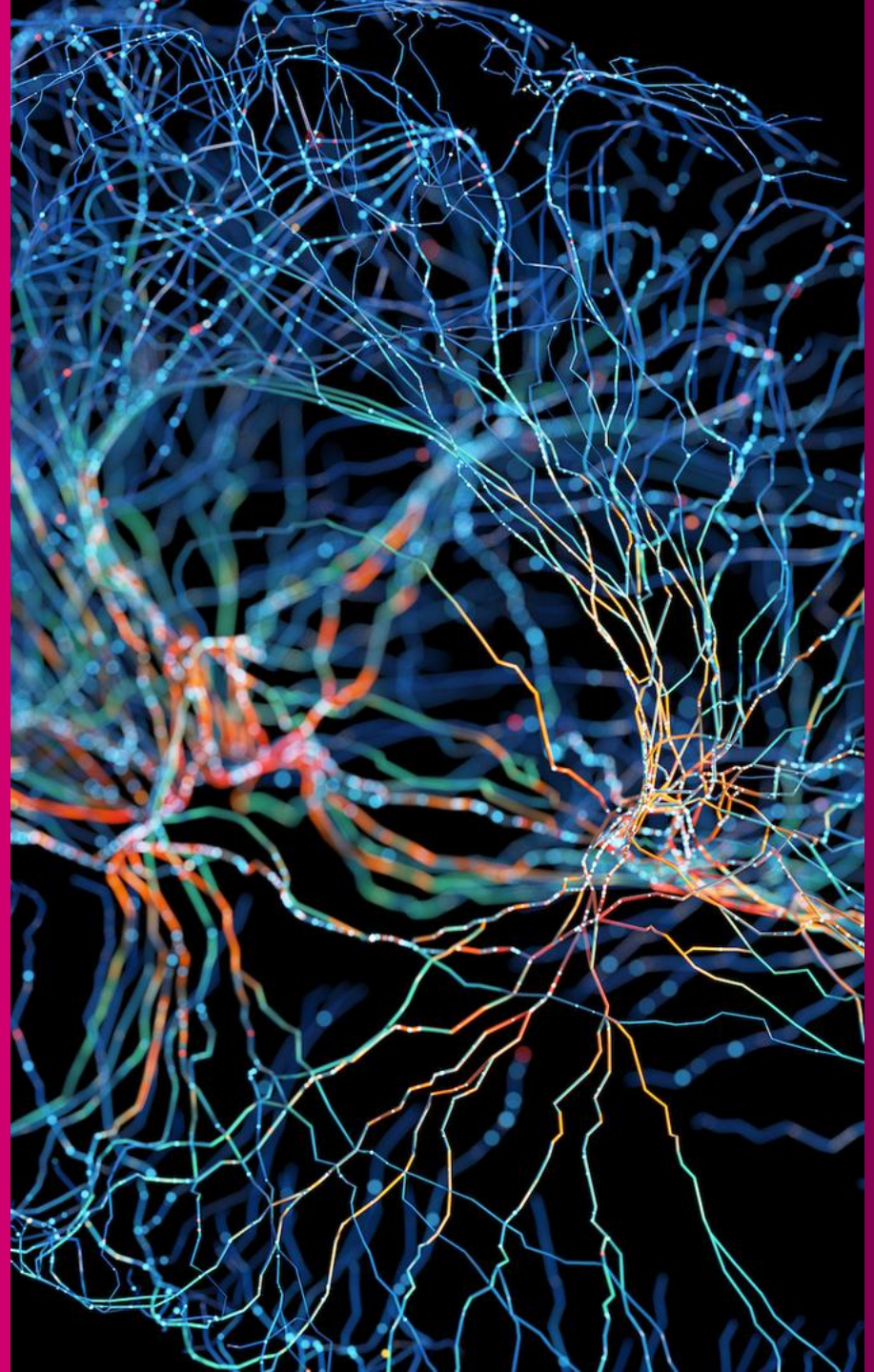


Clinical Trials Appendix

H1 2025 Results Update

29 July 2025

Pipeline
at a glance

Across five focus therapy areas:

Oncology

BioPharmaceuticals

CVRM | R&I | V&I

Rare Disease

196

projects in our development pipeline

19

new molecular entities (NME) in our late-stage pipeline

132

new molecular entities (NME) or major lifecycle management (LCM) projects in Phase II and Phase III

24

regulatory approvals in major markets since FY 2024

As of 29 July 2025.
Appendix: Glossary.

Key upcoming pipeline catalysts: 2025 and 2026

Oncology BioPharmaceuticals Rare Disease

	H2 2025	H1 2026	H2 2026
Regulatory decision[1,2]	***Calquence*** – MCL (1L) (ECHO) (JP) ***Datroway*** – HR + HER2- breast cancer (2L+) (TROPION-Breast01) (CN) ***Enhertu*** – HER2-low and ultralow met. breast cancer (DESTINY-Breast06) (JP) ***Imfinzi*** – resectable early-stage gastric and GEJ cancers (MATTERHORN) ***Imfinzi*** – early-stage NSCLC (perioperative) (AEGEAN) (JP) ***Imfinzi*** – muscle-invasive bladder cancer (NIAGARA) JP) ***Lynparza*** – mCRPC (1L) (PROpel) (CN) ***Tezspire*** – CRwNP (WAYPOINT) ***Ultomiris*** – NMOSD (CHAMPION-NMOSD) (CN) ***Koselugo*** – adult NF1-PN (KOMET)	***Calquence*** – CLL (1L fixed duration) (AMPLIFY) (US) ***Enhertu*** – HER2-low and ultralow met. breast cancer (DESTINY-Breast06) (CN) ***Imfinzi + Imjudo*** – NSCLC (1L) (POSEIDON) (CN) ***Imfinzi + Imjudo*** – HCC (1L) (HIMALAYA) (CN) ***Imfinzi*** – endometrial cancer (1L) (DUO-E) (CN) **camizestrant** – *ESR1*m HR+ HER2- met. breast cancer (1L switch) (SERENA-6) ***Fasenra*** – EGPA (Mandara) (CN) ***Tezspire*** – severe asthma (DIRECTION) (CN) ***Wainua*** – ATTRv-PN (NEURO-TTRansform) (CN)	
Key Phase III data readouts	***Datroway*** – met. TNBC (TROPION-Breast02) ***Enhertu*** – high-risk early HER2+ breast cancer (adjuvant) (DESTINY-Breast05) ***Imfinzi*** – muscle-invasive bladder cancer (VOLGA) **ceralasertib** – post-IO NSCLC (LATIFY) ***Fasenra*** – moderate to severe COPD (RESOLUTE) ***Saphnelo*** – moderate to severe SLE (TULIP-SC) ***Ultomiris*** – HSCT-TMA (TMA-313) **efzimfotase alfa** – hypophosphatasia (HICKORY/CHESTNUT)	***Datroway + Imfinzi*** – non-squamous/non-squamous TROP2+ NSCLC (1L) (AVANZAR) ***Datroway*** – non-squamous NSCLC (1L) (TROPION-Lung07) ***Imfinzi*** – locoregional HCC (EMERALD-3) ***Tagrisso + Orpathys*** – *EGFR*m NSCLC (2L) (SAFFRON) **sonesitatug vedotin (AZD0901)** – CLDN18.2+ gastric cancer (2L+) (CLARITY-Gastric01) **tozorakimab** – COPD (OBERON/TITANIA) **tozorakimab** – COPD (MIRANDA) ***Ultomiris*** – IgAN (ICAN) **efzimfotase alfa** – hypophosphatasia (MULBERRY)	***Datroway + Tagrisso*** – *EGFR*m NSCLC (2L) (TROPION-Lung15) ***Imfinzi*** – early HCC (EMERALD-2) **camizestrant** – HR+ HER2- met. breast cancer (1L) (SERENA-4) ***Saphnelo*** – lupus nephritis (IRIS) ***Saphnelo*** – systemic sclerosis (DAISY) ***Wainua*** – ATTR-CM (CARDIO-TTRansform) **tozorakimab** – LRTD (TILIA) **Ultomiris** – CSA-AKI (ARTEMIS)

Key upcoming pipeline catalysts are defined by a threshold of non-risk adjusted global peak year revenue expectations as of 29 July 2025

[1]Regulatory decision includes programmes under review in a major market

[2]Inclusion dependent on status of regulatory submission and/or submission acceptance in regions in which submission acceptance is granted

As of 29 July 2025.

Appendix: Glossary.

Clinical Trials Appendix: selected highlights

	BioPharmaceuticals	Oncology	Rare Disease
Approved medicines: key LCM	AIRSUPRA™ (albuterol 90 mcg/budesonide 80 mcg) Inhalation Aerosol Saphnelo™ (anifrolumab-fnia) Intravenous Use 300 mg/vial BREZTRI AEROSPHERE™ TEZSPIRE™ (tezepelumab-ekko) Subcutaneous Injection 210 mg Fasenra® (benralizumab) Subcutaneous Injection 30 mg WAINUA™ (eplontersen)	TAGRISSO® osimertinib ENHERTU® DATROWAY® datopotamab deruxtecan-dlnk CALQUENCE® (acalabrutinib) 100 mg capsules Lynparza™ olaparib IMFINZI® durvalumab Injection for Intravenous Use 50 mg/mL IMJUDO® tremelimumab-actl Injection for Intravenous Use 20 mg/mL Truqap™ capivasertib 160 mg • 200 mg tablets	ULTOMIRIS® (ravulizumab) injection for intravenous use Koselugo® (selumetinib) 10 mg & 25 mg capsules
Next-wave pipeline: registrational studies ongoing	balcinrenone/dapagliflozin (MRM/SGLT2) baxdrostat (aldosterone synthase inhibitor) baxdrostat/dapagliflozin (ASI/SGLT2) zibotentan/dapagliflozin (ETA receptor antagonist/SGLT2) tozorakimab (IL-33 ligand mAb)	camizestrant (next generation oral SERD) saruparib (PARP1 inhibitor) sonesitatug vedotin (AZD0901, CLDN18.2 ADC) surovatamig (AZD0486, CD19/CD3 TCE) rilvegostomig (PD-1/TIGIT bispecific) volrustomig (PD-1/CTLA-4 bispecific)	cliramitug (ALXN2220, TTR depleter) efzimfotase alfa (enzyme replacement therapy) eneboparatide (PTH 1 agonist) gefurulimab (C5 inhibitor)

As of 29 July 2025.
Appendix: Glossary.

Project movements since Q1 2025 update

New to Phase I

NME

AZD0240
KRAS G12D armoured TCR-T solid tumours

AZD1613
inflammation and metabolism modulation kidney disease

AZD4248
cellular health cardiorenal disease

AZD4360
CLDN18.2 ADC solid tumours

AZD4954
Lp(a) inhibitor dyslipidaemia

Additional indication

AZD5492
CD20 TITAN T-cell engager systemic lupus erythematosus

volrustomig eVOLVE-RCC02
PD-1/CTLA-4 bispecific mAb 1L advanced clear cell renal cell carcinoma (ccRCC)

New to Phase II

NME

AZD0120
autologous anti-CD19 and anti-BCMA CAR-T cell immunotherapy multiple myeloma

AZD6793
IRAK4 inhibitor COPD

tilatamig samrotecan (AZD9592)
EGFR/cMET TOP1i ADC solid tumours

New to pivotal trial

NME

laroprovstat (AZD0780) AZURE
PCSK9 inhibitor dyslipidaemia

Additional indication

rilvegostomig + *Imjudo* ARTEMIDE-HCC01#
PD-1/TIGIT bispecific mAb + CTLA-4 + VEGFi 1L HCC

Life-cycle Management

***Ultomiris* AWAKE**
anti-C5 mAb delayed graft function

New to registration

NME

camizestrant + CDK4/6i SERENA-6
selective estrogen receptor degrader + CDK4/6 inhibitor 1st-line HR+ HER2- ESR1m breast cancer

Life-cycle Management

***Enhertu* DESTINY-Gastric04#**
HER2 targeting ADC 2nd-line HER2+ gastric cancer

***Imfinzi* + FLOT MATTERHORN#**
PD-L1 mAb + CTx neoadjuvant/adjuvant gastric cancer

Phase progressions based on first subject in achievement
Partnered and/or in collaboration
As of 29 July 2025.
Appendix: Glossary.

Project movements since Q1 2025 update

Removed from Phase I	Removed from Phase II	Removed from Phase III	Approved/removed from registration
AZD5851 GPC3 CAR-T hepatocellular carcinoma **AZD6422** CLDN18.2 CAR-T solid tumours **NT-125** autologous, fully-individualised, multi-specific TCR-T targeting neoantigens solid tumours	**Additional indication** **zibotentan/dapagliflozin** endothelin A receptor antagonist/SGLT2 inhibitor liver cirrhosis **Life-cycle management** ***Ultomiris*** anti-C5 mAb proliferative lupus nephritis		

Phase progressions based on first subject in achievement
Partnered and/or in collaboration
As of 29 July 2025.
Appendix: Glossary.

Q2 2025 Oncology new molecular entity[1] pipeline

Phase I

22 New Molecular Entities

- surovatamig (AZD0486) CD19/CD3 TCE r/r B-cell non-Hodgkin lymphoma
- surovatamig (AZD0486) SYRUS CD19/CD3 TCE B-cell acute lymphoblastic leukaemia
- volrustomig + lenvatinib PD-1/CTLA-4 bispecific mAb + VEGF advanced RCC
- AZD0305 GPRC5D ADC relapsed/refractory multiple myeloma
- AZD1390 ATM inhibitor glioblastoma
- AZD2284 STEAP2 radioconjugate prostate cancer
- AZD4360 CLDN18.2 ADC solid tumours
- AZD5863 CLDN18.2 x CD3 bispecific antibody (HBM7022) solid tumours
- AZD8421 CDK2 inhibitor solid tumours
- AZD9829 CD123 TOP1i ADC AML, MDS
- NT-175 TGFBR2 KO armored TCR-T targeting TP53 R175H solid tumours
- surovatamig (AZD0486) SOUNDTRACK-E CD19/CD3 next-generation bispecific T-cell engager mature B-cell malignancies
- volrustomig eVOLVE-RCC02 PD-1/CTLA-4 bispecific mAb 1L advanced clear cell renal cell carcinoma
- AZD0022 KRas G12D inhibitor solid tumours
- AZD0240 KRAS G12D armoured TCRT solid tumours
- AZD0754 STEAP2 CAR-T prostate cancer
- AZD2068 EGFR cMET radioconjugate solid tumours
- AZD3470 PRMT5 inhibitor classic Hodgkin lymphoma, solid tumours
- AZD5492 CD20 TITAN T-cell engager haematology
- AZD7003 (China) GPC3 CAR-T hepatocellular carcinoma/squamous NSCLC
- AZD9793 GPC3 T-cell engager solid tumours
- NT-112 TGFBR2 KO armored TCR-T targeting KRAS G12D solid tumour

Phase II

17 New Molecular Entities

- camizestrant SERD HR+ breast cancer
- FPI-2265# PSMA radioconjugate prostate cancer
- puxitatug samrotecan B7-H4 targeting ADC solid tumours
- saruparib PARP1Sel solid tumours
- surovatamig (AZD0486) SOUNDTRACK-B CD19/CD3 TCE B-cell non-Hodgkin lymphoma
- volrustomig eVOLVE-01 PD-1/CTLA-4 bispecific mAb NSCLC
- volrustomig CANTOR PD-1/CTLA-4 bispecific mAb colorectal cancer (mCRC)
- AZD0120 autologous anti-CD19 and anti-BCMA CAR-T cell immunotherapy multiple myeloma
- AZD9574 PARP1Sel advanced solid malignancies
- ceralasertib ATR inhibitor solid tumours
- IPH5201 + *Imfinzi*# CD39 + PD-L1 neoadjuvant/adjuvant NSCLC
- rilvegostomig ARTEMIDE-01# PD-1/TIGIT bispecific mAb solid tumours
- sonesitatug vedotin (AZD0901) CLDN18.2 MMAE ADC solid tumours
- tilatamig samrotecan (AZD9592) EGFR/cMET TOP1i ADC solid tumours
- volrustomig PD-1/CTLA-4 bispecific mAb solid tumours
- volrustomig eVOLVE-02 PD-1/CTLA-4 bispecific mAb cervical cancer, head and neck squamous cell carcinoma
- AZD5335 anti-FRα TOP1i ADC ovarian cancer, lung adenocarcinoma

Phase III

19 New Molecular Entities

- *Imfinzi* +/- oleclumab +/- monalizumab PACIFIC-9# PD-L1+NKG2A or PD-L1+CD73 unresectable stage III NSCLC
- camizestrant +/- abemaciclib CAMBRIA-2 SERD ER+/HER2- early breast cancer
- ceralasertib + *Imfinzi* LATIFY ATR inhibitor + PDL-1 NSCLC
- rilvegostomig ARTEMIDE-Lung03# PD-1/TIGIT bispecific mAb non-squamous NSCLC 1L
- rilvegostomig + *Enhertu* ARTEMIDE-Gastric01# PD-1/TIGIT bispecific mAb + HER2 targeting ADC 1L HER2+ gastric cancer
- rilvegostomig ARTEMIDE-Biliary01# PD-1/TIGIT bispecific mAb adjuvant biliary tract cancer
- saruparib EvoPAR-Prostate01 PARP1Sel metastatic castration-sensitive prostate cancer
- surovatamig (AZD0486) SOUNDTRACK-F1 CD19/CD3 TCE follicular lymphoma
- volrustomig eVOLVE-Lung02 PD-1/CTLA-4 bispecific mAb 1L metastatic NSCLC
- volrustomig eVOLVE-Meso PD-1/CTLA-4 bispecific mAb 1L unresectable malignant pleural mesothelioma
- camizestrant + palbociclib SERENA-4 SERD+CDK4/6i 1L HR+ HER2- breast cancer
- camizestrant CAMBRIA-1 SERD HR+ HER2- extended adjuvant breast cancer
- rilvegostomig ARTEMIDE-Lung02# PD-1/TIGIT bispecific mAb squamous NSCLC 1L
- rilvegostomig + *Imjudo* ARTEMIDE-HCC01# PD-1/TIGIT bispecific mAb + CTLA-4 + VEGFi 1L HCC
- rilvegostomig ARTEMIDE-Lung04# PD-1/TIGIT bispecific mAb NSCLC 1L, PDL1 >/=50
- saruparib EvoPAR-Breast01 PARP1Sel BRCA/PALB2m HR+ve metastatic breast cancer
- sonesitatug vedotin CLARITY-Gastric01 CLDN18.2 MMAE ADC gastric 2L+
- volrustomig eVOLVE-Cervical PD-1/CTLA-4 bispecific mAb locally advanced cervical cancer
- volrustomig eVOLVE-HNSCC PD-1/CTLA-4 bispecific mAb unresected locally advanced HNSCC

Under review

1 New Molecular Entity

- camizestrant + CDK4/6i SERENA-6 SERD+CDK4/6i 1L HR+ HER2- ESR1m breast cancer

Phase progressions based on first subject in achievement
1. Includes additional indications for assets where the lead is not yet launched
Partnered and/or in collaboration
As of 29 July 2025.
Appendix: Glossary.

Q2 2025 Oncology lifecycle management[1] pipeline

Phase I

0 Projects

Phase II

9 Projects

- *Enhertu* (platform) DESTINY-Breast07#
  HER2 targeting ADC HER2+ breast cancer
- *Enhertu* DESTINY-PanTumor03#
  HER2 targeting ADC HER2 expressing solid tumours
- *Enhertu* DESTINY-PanTumour01#
  HER2 ADC HER2 mutant tumours
- *Imfinzi* (platform) BEGONIA
  PD-L1 mAb with paclitaxel and multiple novel oncology therapies 1L metastatic TNBC
- *Imfinzi* (platform) HUDSON
  PD-L1+multiple novel ONC therapies post IO NSCLC
- *Imfinzi* (platform) NeoCOAST-2#
  PD-L1 mAb + multiple novel oncology therapies NSCLC
- *Tagrisso + Orpathys* SAVANNAH#
  EGFR+MET advanced EGFRm NSCLC
- *Tagrisso* ORCHARD platform study#
  EGFR+multiple novel ONC therapies 2L EGFRm osimertinib-resistant NSCLC
- *Truqap*
  AKT prostate cancer

Phase III

35 Projects

- *Calquence* + R-CHOP ESCALADE
  BTK+R-CHOP 1L DLBCL
- *Datroway* + pembrolizumab TROPION-Lung07#
  TROP2 ADC 1L NSCLC PD-L1 <50% non-squamous
- *Datroway* + rilvegostomig TROPION-Lung12 #
  TROP2 ADC + PD-1/TIGIT Stage I adenocarcinoma NSCLC who are ctDNA-positive or have high-risk pathological features
- *Datroway* TROPION-Breast02#
  TROP2 ADC 1st-line triple negative breast cancer
- *Enhertu* DESTINY-Breast05#
  HER2 ADC HER2+ post-neoadjuvant high-risk breast cancer
- *Enhertu* DESTINY-Endometrial01#
  HER2 targeting ADC + PD-1/TIGIT bispecific mAb endometrial cancer
- *Imfinzi* + CRT KUNLUN
  PD-L1+CRT locally-advanced ESCC
- *Imfinzi* + VEGF EMERALD-2
  PD-L1+VEGF adjuvant HCC
- *Imfinzi* + VEGF + TACE EMERALD-1
  PD-L1+VEGF+TACE locoregional HCC
- *Lynparza* MONO-OLA1#
  PARP 1L BRCAwt ovarian cancer
- *Tagrisso* +/- CTx neoadjuvant NeoADAURA
  EGFR+/-CTx Stage II/III resectable EGFRm NSCLC
- *Truqap* + abiraterone CAPItello-281
  AKT+abiraterone PTEN deficient mHSPC
- *Tagrisso* ADAURA2
  EGFR adjuvant EGFRm NSCLC stage Ia2-Ia3 following complete tumour resection
- *Datroway + Imfinzi* + CTx AVANZAR#
  TROP2 ADC + PD-L1 + CTx non-squamous or non-squamous TROP2+ NSCLC 1L
- *Datroway + Imfinzi* TROPION-Breast05#
  TROP2 ADC + PD-L1 1st-line triple negative breast cancer
- *Datroway + Tagrisso* TROPION-Lung14#
  TROP2 ADC + EGFR inhibitor 1L EGFRm NSCLC
- *Datroway* +/- *Imfinzi* TROPION-Breast03#
  TROP2 ADC +/- PD-L1 adjuvant residual disease TNBC
- *Enhertu* + rilvegostomig DESTINY-BTC01#
  HER2 targeting ADC + PD-1/TIGIT bispecific mAb 1L HER2+ biliary tract cancer
- *Enhertu* DESTINY-Breast11#
  HER2 ADC neoadjuvant HER2+ breast cancer
- *Imfinzi* + domvanalimab (AB154) PACIFIC-8#
  PD-L1+TIGIT+CTx unresectable stage III NSCLC
- *Imfinzi + Imjudo* + SoC NILE
  PD-L1+CTLA-4+SoC 1L urothelial cancer
- *Imfinzi* POTOMAC
  PD-L1 non-muscle invasive bladder cancer
- *Lynparza + Imfinzi* + bevacizumab DUO-O#
  PARP+PD-L1+VEGF 1L ovarian cancer
- *Orpathys + Imfinzi* SAMETA#
  MET+PD-L1 1L papillary renal cell carcinoma
- *Tagrisso + Orpathys* SAFFRON#
  EGFR + MET advanced EGFRm non-small cell lung cancer
- *Truqap + Faslodex* + palbociclib CAPItello-292
  AKT+fulvestrant+CDK4/6 1L triplet in early relapse/ET resistant locally advanced or mBC
- *Datroway + Imfinzi* TROPION-Breast04#
  TROP2 ADC + PD-L1 neoadjuvant/adjuvant triple negative or HR-low/HER2-negative breast cancer
- *Datroway* + rilvegostomig TROPION-Lung10# TROP2 ADC locally advanced or metastatic non-squamous NSCLC with high PD-L1 expression (TC ≥50%) and without actionable genomic alterations
- *Datroway + Tagrisso* TROPION-Lung15#
  TROP2 ADC + EGFR inhibitor 2L advanced or metastatic EGFRm NSCLC
- *Datroway* TROPION-Lung08#
  TROP2 ADC 1L metastatic NSCLC
- *Enhertu* DESTINY-Breast09#
  HER2 ADC HER2+ breast cancer 1L
- *Enhertu* DESTINY-Lung04#
  HER2 ADC HER2m NSCLC 1L
- *Imfinzi* + EV +/- *Imjudo* VOLGA
  PD-L1 + nectin-4 targeting ADC +/- CTLA-4 MIBC
- *Imfinzi + Imjudo* + TACE +/- lenvatinib EMERALD-3
  PD-L1+CTLA4+VEGF+/-chemoembolization locoregional HCC
- *Imfinzi* post-SBRT PACIFIC-4#
  PD-L1 mAb post-SBRT stage I/II NSCLC

Under review

3 Projects

- *Enhertu* DESTINY-Gastric04#
  HER2 ADC HER2+ gastric 2L
- *Imfinzi* + CRT PACIFIC-5 (China)#
  PD-L1+CRT locally-advanced stage III NSCLC
- *Imfinzi* + FLOT MATTERHORN#
  PD-L1+CTx neoadjuvant/adjuvant gastric cancer

Phase progressions based on first subject in achievement
1. Includes significant lifecycle management projects and parallel indications for assets beyond Phase III
Partnered and/or in collaboration
As of 29 July 2025.
Appendix: Glossary.

Q2 2025 BioPharmaceuticals new molecular entity[1] pipeline

Phase I

15 New Molecular Entities

- AZD0120
  autologous anti-CD19 and anti-BCMA CAR-T cell immunotherapy systemic lupus erythematosus
- AZD0233
  CX3CR1 dilated cardiomyopathy
- AZD0292
  pseudomonas Psl-PcrV bispecific mAb non-CF bronchiectasis
- AZD1163
  bispecific antibody rheumatoid arthritis
- AZD1613
  inflammation and metabolism modulation kidney disease
- AZD1705
  lipid lowering cardiovascular disease
- AZD4144
  inflammation modulator cardiorenal disease
- AZD4248
  cellular health cardiorenal disease
- AZD4954
  Lp(a) inhibitor dyslipidaemia
- AZD5148
  anti-clostridioides difficile TcdB mAb reduction of *C.diff* recurrence
- AZD5492
  CD20 TITAN T-cell engager systemic lupus erythematosus
- AZD6912
  siRNA rheumatoid arthritis
- AZD7760
  mAb combination targeting S aureus virulence factors prevention of Staph aureus infection
- AZD8965
  inhibition of arginase enzyme idiopathic pulmonary fibrosis
- mRNA VLP vaccine
  mRNA-VLP vaccine prevention of COVID-19

Phase II

15 New Molecular Entities

- atuliflapon
  FLAP asthma
- balcinrenone/dapagliflozin
  MR modulator + SGLT2 inhibitor CKD
- opemalirsen (AZD2373)
  podocyte health nephropathy
- tozorakimab
  IL-33 asthma
- AZD2389
  anti-fibrotic mechanism metabolic dysfunction-associated steatohepatitis
- AZD2693
  NASH resolution non-alcoholic steatohepatitis
- AZD3427
  relaxin mimetic heart failure
- AZD4604
  inhaled JAK1 inhibitor asthma
- AZD5004
  oral GLP-1 receptor agonist T2D/chronic weight management
- AZD5462#
  RXFP1 agonist heart failure
- AZD6234
  peptide chronic weight management in overweight or obesity
- AZD6793
  IRAK4 inhibitor inflammatory diseases
- AZD7798
  humanised monoclonal antibody targets T-cells subset Crohn's disease
- AZD8630#
  inhaled TSLP FAb asthma
- AZD9550 + AZD6234
  GLP-1R glucagon dual agonist obesity

Phase III

8 New Molecular Entities

- balcinrenone/dapagliflozin
  MR modulator + SGLT2 inhibitor heart failure with CKD
- baxdrostat BaxHTN
  aldosterone synthase inhibitor hypertension
- baxdrostat/dapagliflozin
  aldosterone synthase inhibitor and reversible inhibitor of SGLT2 CKD
- baxdrostat/dapagliflozin
  aldosterone synthase inhibitor and reversible inhibitor of SGLT2 prevention of heart failure
- laroprovstat (AZD0780) AZURE
  PCSK9 dyslipidemia
- tozorakimab OBERON TITANIA PROSPERO MIRANDA
  IL-33 COPD
- tozorakimab TILIA
  IL-33 severe viral lower respiratory tract disease
- zibotentan/dapagliflozin
  endothelin A receptor antagonist/SGLT2i CKD with high proteinuria

Under review

0 New Molecular Entities

Phase progressions based on first subject in achievement
1. Includes additional indications for assets where the lead is not yet launched
Partnered and/or in collaboration
As of 29 July 2025.
Appendix: Glossary.

Q2 2025 BioPharmaceuticals life cycle management[1] pipeline

Phase I
0 Projects

Phase II
0 Projects

Phase III
13 Projects

- *Breztri*/Trixeo (PT010) KALOS LOGOS
  LABA/LAMA/ICS asthma
- *Breztri*/Trixeo THARROS#
  LABA/LAMA/ICS cardiopulmonary outcomes trial in COPD
- *Fasenra* RESOLUTE#
  IL-5R chronic obstructive pulmonary disease
- *Saphnelo* IRIS#
  type I IFN receptor mAb lupus nephritis
- *Saphnelo* LAVENDER#
  type I IFN receptor mAb cutaneous lupus erythematosus
- *Tezspire* CROSSING#
  TSLP mAb eosinophilic oesophagitis
- *Wainua*#
  ligand-conjugated antisense ATTR-cardiomyopathy
- *Breztri*/Trixeo ATHLOS
  LABA/LAMA/ICS COPD cardiopulmonary exercise trial
- *Fasenra* NATRON
  IL-5R hypereosinophilic syndrome
- *Saphnelo* DAISY#
  type I IFN receptor systemic sclerosis
- *Saphnelo* JASMINE#
  type I IFN receptor mAb myositis
- *Saphnelo* TULIP-SC#
  type I IFN receptor systemic lupus erythematosus (subcutaneous)
- *Tezspire* EMBARK, JOURNEY#
  TSLP chronic obstructive pulmonary disease

Under review
1 Project

- *Tezspire* WAYPOINT#
  TSLP nasal polyps

Phase progressions based on first subject in achievement
1. Includes significant lifecycle management projects and parallel indications for assets beyond Phase III
Partnered and/or in collaboration
As of 29 July 2025.
Appendix: Glossary.

Q2 2025 Rare Disease pipeline[1]

Phase 1
3 Projects

- ALXN1920
  kidney-targeted factor H fusion protein nephrology
- ALXN2030
  siRNA targeting complement C3 antibody-mediated rejection
- ALXN2080
  oral factor D healthy volunteers

Phase 2
1 Project

- MEDI1341#
  alpha synuclein mAb multiple system atrophy/Parkinson's disease

Phase 3
9 Projects

- anselamimab
  fibril-reactive mAb amyloid light-chain amyloidosis
- cliramitug (ALXN2220) DepleTTR-CM#
  TTR depleter transthyretin amyloid cardiomyopathy
- efzimfotase alfa
  next generation TNSALP ERT hypophosphatasia
- eneboparatide CALYPSO
  parathyroid hormone receptor 1 hypoparathyroidism
- gefurulimab PREVAIL
  novel anti-C5, dual binding, nanobody generalised myasthenia gravis
- *Ultomiris*
  anti-complement C5 mAb haematopoietic stem cell transplant–associated thrombotic microangiopathy
- *Ultomiris* ARTEMIS
  anti-complement C5 mAb cardiac surgery-associated acute kidney injury
- *Ultomiris* AWAKE
  anti-complement C5 mAb delayed graft function
- *Ultomiris* I CAN
  anti-complement C5 mAb immunoglobulin A nephropathy

Reg
1 Project

- *Koselugo* KOMET#
  MEK inhibitor neurofibromatosis type 1 adult

Phase progressions based on first subject in achievement
1. Includes new molecular entities and significant lifecycle management projects
Partnered and/or in collaboration
As of 29 July 2025.
Appendix: Glossary.

Active designations in our pipeline

2 Priority Review

- *Imfinzi* resectable early-stage gastric and GEJ cancers MATTERHORN (US)
- cliramitug (ALXN2220) DepleTTR-CM (JP)

6 Breakthrough / PRIME[1] / Sakigake[2]

- *Tezspire* COPD EMBARK, JOURNEY (US)
- tozorakimab severe viral LRTD TILIA (CN)
- camizestrant 1L HR+ HER2- *ESR1*m breast cancer SERENA-6 (US)
- *Enhertu* HER2+ breast cancer 1L DESTINY-Breast09 (US)
- *Imfinzi* resectable early-stage gastric and GEJ cancers MATTERHORN (US)
- *Ultomiris* HSCT-TMA paed (US)

12 Fast Track

- AZD0292 Psl-PcrV N3Y NCFBE (US)
- AZD3427 relaxin mimetic heart failure (US)
- AZD7760 Staph aureus mAbs-Hemodialysis (US)
- balci/dapa HF with CKD (US)
- tozorakimab COPD (US)
- tozorakimab severe viral LRTD (US)
- *Wainua* ATTR-Cardiomyopathy (US)
- camizestrant 1L HR+ HER2- *ESR1*m breast cancer SERENA-6 (US)
- *Orpathys* + *Tagrisso* NSCLC SAVANNAH/SAFFRON (US)
- ALXN2220 DepleTTR-CM (US)
- anselamimab AL amyloidosis CAEL101-301/2 (US)
- eneboparatide HypoPT (US)

3 Qualified infectious disease product

- AZD0292 Psl-PcrV N3Y NCFBE (US)
- AZD5148 C. difficile mAb - Prevention of Recurrence (US)
- AZD7760 prevention of Staph aureus infection (US)

19 Orphan

- *Fasenra* HES NATRON (US)
- *Saphnelo* myositis JASMINE (US)
- *Saphnelo* systemic sclerosis (US)
- *Tezspire* EoE CROSSING (US)
- *Wainua* transthyretin-mediated amyloidosis (US)
- cliramitug (ALXN2220) DepleTTR-CM (US)
- cliramitug (ALXN2220) DepleTTR-CM (EU)
- cliramitug (ALXN2220) DepleTTR-CM (JP)
- anselamimab AL amyloidosis CAEL101-301/2 (US)
- anselamimab AL amyloidosis CAEL101-301/2 (EU)
- eneboparatide HypoPT (EU)
- eneboparatide HypoPT (US)
- gefurulimab myasthenia gravis PREVAIL (US)
- *Koselugo* NF1 adult 1L KOMET (US)
- *Koselugo* NF1 adult 1L KOMET (EU)
- *Koselugo* NF1 adult 1L KOMET (JP)
- *Koselugo* NF1 adult 1L KOMET (CN)
- *Ultomiris* HSCT-TMA ALXN1210-TM-313 (US)
- *Ultomiris* HSCT-TMA ALXN1210-TM-313 (JP)

ACCELERATED APPROVAL, these regulations allowed medicines for serious conditions that addressed an unmet medical need to be approved based on a surrogate endpoint

BREAKTHROUGH DESIGNATION is a process designed to expedite the development and review of medicines which may demonstrate substantial improvement over available therapy. [1]PRIME is a scheme launched by the EMA to enhance support for the development of medicines that target an unmet medical need. [2]SAKIGAKE is aimed at early introduction of innovative medicines, medical devices, etc. that are initially developed in Japan

FAST TRACK is a process designed to facilitate the development, and expedite the review of medicines to treat serious conditions and fill an unmet medical need

PRIORITY REVIEW DESIGNATION is the US FDA's goal to take action on an application within 6 months

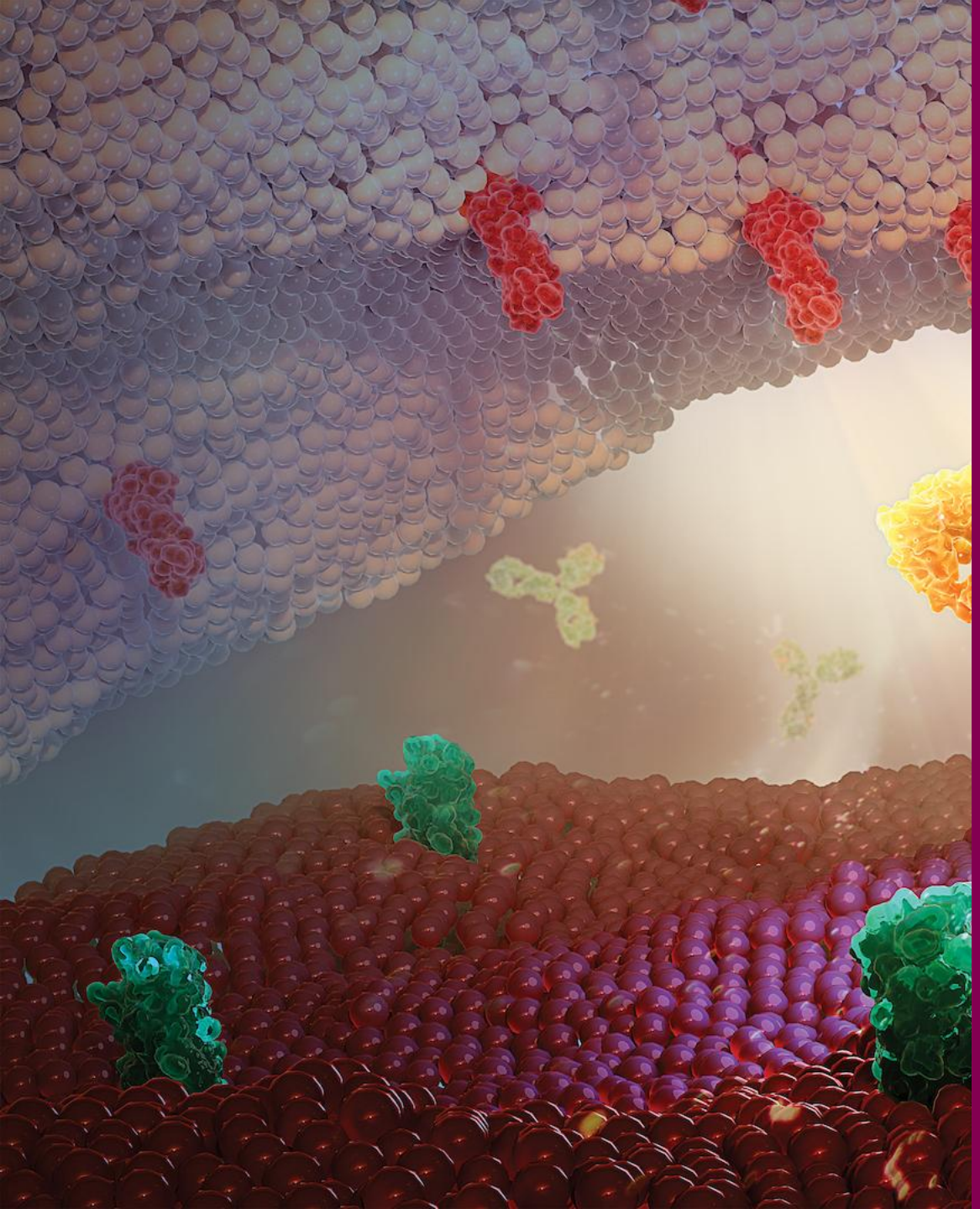
ORPHAN DRUG DESIGNATION, intended for treatment, diagnosis or prevention of rare diseases/disorders that affect fewer than 200,000 patients in the US, or that affect more than 200,000 patients but are not expected to recover the costs of developing and marketing a treatment drug

QUALIFIED INFECTIOUS DISEASE PRODUCT designation confers particular advantages, including priority review by the US Food and Drug Administration (FDA) and fast-track designation, which can accelerate development of a product, as well as an additional five years' market exclusivity if a product is licensed.

Oncology BioPharmaceuticals Rare Disease

As of 29 July 2025.

Appendix: Glossary.



Oncology:
approved medicines and late-stage pipeline

AstraZeneca

Calquence (BTK inhibitor)

Blood cancers

Trial	Population	Patients	Design	Endpoints	Status
Phase III **AMPLIFY (ACE-CL-311)** **NCT03836261**	Previously untreated CLL	981	• Arm 1; *Calquence* + venetoclax • Arm 2: *Calquence* + venetoclax + obinutuzumab • Arm 3: FCR or BR	• Primary endpoint: IRC PFS (Arm 1 vs. Arm 3) • Secondary endpoints: IRC PFS (Arm 2 vs. Arm 3) and INV PFS (Arm 1 vs. Arm 3; Arm 2 vs. Arm 3)	• FPCD: Q1 2019 • LPCD: Q3 2023 • Data readout: Q3 2024 • Primary endpoint met
Phase III **ECHO (ACE-LY-308)** **NCT02972840**	Previously untreated MCL	634	• Arm 1: *Calquence* + bendamustine + rituximab • Arm 2: bendamustine + rituximab	• Primary endpoint: PFS by Lugano Classification for NHL • Secondary endpoints: IA, PFS, ORR, DoR, time to response and OS	• FPCD: Q2 2017 • LPCD: Q1 2023 • Data readout: Q2 2024 • Primary endpoint met
Phase III **ESCALADE** **NCT04529772**	DLBCL	600	• *Calquence* + rituximab, cyclophosphamide, doxorubicin, vincristine and prednisone	• Primary endpoint: PFS	• FPCD: Q1 2021 • Data anticipated: >2026
Phase III **NCT04075292**	Untreated CLL	155	• Arm 1: *Calquence* • Arm 2: chlorambucil + rituximab	• Primary endpoint: PFS • Secondary endpoints: ORR and DoR	• FPCD: Q1 2020 • Data readout: Q2 2024
Phase II **TrAVeRse** **NCT05951959**	Treatment-naïve MCL	100	• Open-label, single-arm trial • *Calquence* + venetoclax + rituximab	• Primary endpoint: MRD-negative CR at end of induction	• FPCD: Q1 2024 • Data anticipated: >2026
Phase Ib **ACE-LY-106** **NCT02717624**	MCL	61	• *Calquence* in combination with bendamustine and rituxumab • Arm 1: treatment naive • Arm 2: R/R • Arm 3: treatment naïve: *Calquence* + venetoclax + rituxumab	• Primary endpoint: safety	• FPCD: Q2 2016 • LPCD: Q2 2022 • Data readout: Q1 2023
Phase I **ACE-LY-003** **NCT02180711**	R/R follicular lymphoma	89	• Arm 1: *Calquence* • Arm 2: *Calquence* + rituximab • Arm 3: *Calquence* + rituximab + lenolidomide	• Primary endpoint: safety	• FPCD: Q1 2015 • Data readout: Q1 2024

As of 29 July 2025.
Appendix: Glossary.

Datroway (datopotamab deruxtecan, TROP2 ADC)

Breast cancer

Trial	Population	Patients	Design	Endpoints	Status
Phase III **TROPION-Breast01** **NCT05104866** **Partnered (Daiichi Sankyo)**	Inoperable or metastatic HR+ HER2- breast cancer after treatment with one or two prior lines of systemic chemotherapy	732	• Open-label, randomised trial • Arm 1: *Datroway* • Arm 2: investigator's choice SoC chemotherapy (eribulin, vinorelbine, capecitabine, gemcitabine)	• Primary endpoints: PFS (BICR) and OS • Secondary endpoints: ORR, DoR, PFS (Inv), DCR, PK parameters and ADA	• FPCD: Q4 2021 • LPCD: Q4 2022 • Data readout: Q3 2023 • Dual primary endpoint (OS) not met
Phase III **TROPION-Breast02** **NCT05374512** **Partnered (Daiichi Sankyo)**	Locally recurrent inoperable or metastatic TNBC	600	• Open-label, randomised trial • Arm 1: *Datroway* • Arm 2: investigator's choice of chemotherapy (paclitaxel, nab-paclitaxel, carboplatin, capecitabine, eribulin mesylate)	• Primary endpoints: PFS (BICR) and OS • Secondary endpoints: PFS (Inv), ORR, DoR, PK parameters and ADA	• FPCD: Q2 2022 • Data anticipated: H2 2025
Phase III **TROPION-Breast03** **NCT05629585** **Partnered (Daiichi Sankyo)**	Stage I-III TNBC without pathological complete response following neoadjuvant therapy	1075	• Open-label, randomised trial • Arm 1: *Datroway* + *Imfinzi* • Arm 2: *Datroway* • Arm 3: investigator's choice of therapy (capecitabine, pembrolizumab, or capecitabine + pembrolizumab)	• Primary endpoint: iDFS • Secondary endpoints: DDFS, OS, PK parameters and ADA	• FPCD: Q4 2022 • Data anticipated: >2026
Phase III **TROPION-Breast04** **NCT06112379** **Partnered (Daiichi Sankyo)**	Perioperative triple-negative or HR-low/HER2-negative breast cancer	1900	• Open-label, randomised • Arm 1: *Datroway* + *Imfinzi* • Arm 2: pembrolizumab + chemotherapy	• Primary endpoint: EFS • Secondary endpoints: pCR, OS, DDFS and safety	• FPCD: Q4 2023 • Data anticipated: >2026
Phase III **TROPION-Breast05** **NCT06103864** **Partnered (Daiichi Sankyo)**	Patients with PD-L1-positive locally recurrent inoperable or metastatic TNBC	625	• Open-label, randomised • Arm 1: *Datroway* + *Imfinzi* • Arm 2: investigator's choice of chemotherapy in combination with pembrolizumab (paclitaxel, nab-paclitaxel, or gemcitabine + carboplatin) • Arm 3: *Datroway*	• Primary endpoint: PFS (BICR) • Secondary endpoints: OS, PFS (inv), ORR, DoR, DCR and safety	• FPCD: Q4 2023 • Data anticipated: >2026

As of 29 July 2025.
Appendix: Glossary.

Datroway (datopotamab deruxtecan, TROP2 ADC)

NSCLC

Trial	Population	Patients	Design	Endpoints	Status
Phase III AVANZAR NCT05687266	1L NSCLC	1280	• Arm 1: *Datroway* + *Imfinzi* + carboplatin • Arm 2: pembrolizumab + CTx •	• Co-primary endpoints: PFS and OS in NSQ ITT and NSQ TROP2 biomarker-positive	• FPCD: Q1 2023 • Data anticipated: H1 2026
Phase III TROPION-Lung01 NCT04656652 Partnered (Daiichi Sankyo)	Previously treated advanced or metastatic NSCLC with or without actionable genomic alterations	590	• Randomised, open-label, parallel assignment • Arm 1: *Datroway* • Arm 2: docetaxel	• Primary endpoints: PFS and OS • Secondary endpoints: ORR, DoR, TTR, DCR, PK parameters and ADA	• FPCD: Q1 2021 • LPCD: Q4 2022 • Data readout: Q3 2023 • Dual primary endpoint met (PFS)
Phase III TROPION-Lung08 NCT05215340 Partnered (Daiichi Sankyo)	Treatment-naïve patients with PD-L1-high advanced or metastatic NSCLC without actionable genomic alterations	740	• Randomised, open-label • Arm 1: *Datroway* + pembrolizumab • Arm 2: pembrolizumab	• Primary endpoints: PFS and OS	• FPCD: Q1 2022 • Data anticipated: H1 2026
Phase III TROPION-Lung07 NCT05555732 Partnered (Daiichi Sankyo)	1L patients with PD-L1 TPS <50% and advanced or metastatic NSCLC without actionable genomic alternations	1170	• Randomised, open-label • Arm 1: *Datroway* + pembrolizumab + platinum chemotherapy • Arm 2: *Datroway* + pembrolizumab • Arm 3: pembrolizumab + pemetrexed + platinum chemotherapy	• Primary endpoints: PFS and OS	• FPCD: Q1 2023 • Data anticipated: H1 2026
Phase III TROPION-Lung10 NCT06357533 Partnered (Daiichi Sankyo)	Locally advanced or metastatic non-squamous NSCLC with high PD-L1 expression (TC ≥50%) and without actionable genomic alterations	675	• Randomised, open-label, sponsor-blinded, parallel assignment • Arm 1: *Datroway* + rilvegostomig • Arm 2: rilvegostomig • Arm 3: pembrolizumab	• Primary endpoints: PFS and OS in TROP2 biomarker-positive participants • Secondary endpoints: PFS and OS in the ITT population, ORR, DoR, TTD, PK parameters, immunogenicity and PFS2	• FPCD: Q2 2024 • Data anticipated: >2026
Phase III TROPION-Lung12 NCT06564844 Partnered (Daiichi Sankyo)	Stage I adenocarcinoma NSCLC who are ctDNA-positive or have high-risk pathological features	660	• Randomised trial • Arm 1: *Datroway* + rilvegostomig • Arm 2: rilvegostomig • Arm 3: standard of care	• Primary endpoint: DFS (BICR) • Secondary endpoint: OS, QoL and PK parameters	• FPCD: Q4 2024 • Data anticipated: >2026

As of 29 July 2025.
Appendix: Glossary.

Datroway (datopotamab deruxtecan, TROP2 ADC)

NSCLC

Trial	Population	Patients	Design	Endpoints	Status
Phase III **TROPION-Lung14** **NCT06350097** **Partnered (Diachii Sankyo)**	EGFRm locally advanced or metastatic NSCLC	562	• Arm 1: *Tagrisso* + *Datroway* • Arm 2: *Tagrisso* monotherapy	• Primary endpoint: PFS (BICR) • Secondary endpoints: OS, PFS by Inv., ORR, DoR; DCR; PFS of CNS met. patients; PFS2; safety; PK parameters and immunogenicity	• FPCD: Q2 2024 • Data anticipated: >2026
Phase III **TROPION-Lung15** **NCT06417814** **Partnered (Daiichi Sankyo)**	Patients with advanced or metastatic EGFRm NSCLC whose disease has progressed on prior Osimertinib	630	• Open-label, sponsor blind, randomised trial • Arm 1: *Datroway* + *Tagrisso* • Arm 2: *Datroway* • Arm 3: Platinum-based doublet CTx	• Dual primary endpoints: PFS (BICR) monotherapy vs. CTx and PFS (BICR) combination vs. CTx • Secondary endpoints: OS, CNS PFS, PFS (Inv.), PFS2, ORR, DoR, DCR, TTR, safety and PRO	• FPCD: Q4 2024 • Data anticipated: H2 2026
Phase II **TROPION-Lung05** **NCT04484142** **Partnered (Daiichi Sankyo)**	Advanced or metastatic NSCLC with actionable genomic alterations and progressed on or after kinase inhibitor therapy and platinum-based chemotherapy	137	• Single-arm, open-label • *Datroway*	• Primary endpoint: ORR • Secondary endpoints: DOR, PFS, OS, safety, PK parameters and ADA	• FPCD: Q1 2021 • LPCD: Q1 2022 • Data readout: Q1 2023
Phase I **TROPION-Lung02** **NCT04526691** **Partnered (Daiichi Sankyo)**	Advanced or metastatic NSCLC	145	• Open-label, two-part (dose escalation and dose expansion), sequential assignment • *Datroway* + pembrolizumab +/- platinum chemotherapy	• Primary endpoints: DLT and safety • Secondary endpoints: ORR, DOR, PFS, OS, PK parameters and ADA	• FPCD: Q4 2020 • LPCD: Q2 2023 • Data readout: Q4 2024
Phase I **TROPION-Lung04** **NCT04612751** **Partnered (Daiichi Sankyo)**	Advanced or metastatic NSCLC	371	• Open-label, two-part (dose escalation, dose expansion), sequential assignment • *Datroway* + *Imfinzi* +/- platinum chemotherapy • Cohort 1 & 2: *Datroway* + *Imfinzi* • Cohort 3 & 4: *Datroway* + *Imfinzi* + carboplatin • Cohort 4a: *Datroway* + *Imfinzi* + carboplatin (SQ 1L only) • Cohort 5 & 6: *Datroway* + rilvegostomig • Cohort 7 & 8: *Datroway* + rilvegostomig + carboplatin • Cohort 9 & 10: *Datroway* + volrustomig + carboplatin • Cohort 11: *Datroway* + volrustomig • Cohort 12, 13 & 14: *Datroway* + sabestomig	• Primary endpoints: DLT and safety • Secondary endpoints: ORR, DOR, PFS, OS, PK parameters and ADA	• FPCD: Q1 2021 • Data anticipated: H1 2026

As of 29 July 2025.
Appendix: Glossary.

Datroway (datopotamab deruxtecan, TROP2 ADC)

Other cancers

Trial	Population	Patients	Design	Endpoints	Status
Phase II **TROPION-PanTumor03** **NCT05489211** **Partnered (Daiichi Sankyo)**	Endometrial cancer, gastric cancer, mCRPC, ovarian cancer, CRC, bladder cancer and BTC	556	• Sub-study 1 (endometrial cancer); • Sub-study 1a: *Datroway* monotherapy • Sub-study 2 (gastric cancer); • Sub-study 2a: *Datroway* + capecitabine • Sub-study 2b: *Datroway* + 5-fluorouracil • Sub-study 3 (mCRPC); • Sub-study 3a: *Datroway* (post-NHA) • Sub-study 3c: *Datroway* + prednisone/prednisolone • Sub-study 4 (ovarian cancer); • Sub-study 4a: *Datroway* • Sub-study 4a (expansion): *Datroway* PSR/PRR (2-3L) • Sub-study 4c: *Datroway* + carboplatin + bevacizumab PSR (2-3L) • Sub-study 5 (CRC); • Sub-study 5a1: *Datroway* (TROP2+ 3L+) • Sub-study 5a2: *Datroway* (TROP2+ 2L+) • Sub-study 5b: *Datroway* + 5-FU/leucovorin or Capecitabine + bevacizumab (TROP2+ 1L) • Sub-study 6 (bladder); • Sub-study 6d: *Datroway* (2L+) • Sub-study 6b: 1L cis-ineligible/2L *Datroway* + rilvegostomig (1L) • Sub-study 6c: post-pembro/EV - *Datroway* + carbo/cisplatin (2L) • Sub-study 7 (BTC) • Sub-study 7a: TROP2+ *Datroway* (2L+)	• Primary endpoints: ORR and safety	• FPCD: Q3 2022 • Data anticipated: >2026
Phase I/II **TROPION-PanTumor02** **NCT05460273** **Partnered (Daiichi Sankyo)**	NSCLC and TNBC and other solid tumours in Chinese patients	119	• Single-arm, multi-cohort trial with no blinding • *Datroway* • China only	• Primary endpoint: ORR • Secondary endpoints: DoR, DCR, BOR, TTR PFS and OS	• FPCD: Q3 2022 • LPCD: Q2 2023 • Data readout: Q2 2024
Phase I **TROPION-PanTumor01** **NCT03401385** **Partnered (Daiichi Sankyo)**	Subjects with advanced solid tumours: NSCLC, TNBC, HR+ breast cancer, HER2-negative gastric/GEJ, oesophageal, urothelial, SCLC, CRPC, PDAC, HNSCC, HR+ HER2-low breast cancer and HER2-positive breast cancer	890	• Open-label, two-part (dose escalation, dose expansion), sequential assignment • *Datroway* • US and Japan	• Primary endpoints: DLT and safety • Secondary endpoints: PK parameters, anti-tumour activity and ADA	• FPCD: Q1 2018 • Data anticipated: H2 2025

As of 29 July 2025.
Appendix: Glossary.

Enhertu (trastuzumab deruxtecan, HER2 ADC)

Breast cancer

Trial	Population	Patients	Design	Endpoints	Status
Phase III **DESTINY-Breast02** **NCT03523585** **Partnered (Daiichi Sankyo)**	HER2-positive, unresectable and/or metastatic breast cancer pretreated with prior SoC HER2 therapies including trastuzumab emtansine	600	• Randomised, open-label, parallel assignment • Arm 1: *Enhertu* • Arm 2: physician's choice of lapatinib + capecitabine or trastuzumab + capecitabine	• Primary endpoint: PFS • Secondary endpoints: OS, ORR, DoR and CBR	• FPCD: Q3 2018 • LPCD: Q4 2020 • Data readout: Q3 2022 • Primary endpoint met
Phase III **DESTINY-Breast03** **NCT03529110** **Partnered (Daiichi Sankyo)**	HER2-positive, unresectable and/or metastatic breast cancer previously treated with trastuzumab and taxane	524	• Randomised, open-label, parallel assignment • Arm 1: *Enhertu* • Arm 2: ado-trastuzumab emtansine	• Primary endpoint: PFS • Secondary endpoints: OS, ORR, DoR and CBR	• FPCD: Q3 2018 • LPCD: Q2 2020 • Data readout: Q3 2021 • Primary endpoint met
Phase III **DESTINY-Breast04** **NCT03734029** **Partnered (Daiichi Sankyo)**	HER2-low, unresectable and/or metastatic breast cancer	557	• Randomised, open-label, parallel assignment • Arm 1: *Enhertu* • Arm 2: physician's choice of SoC chemotherapy (choice of capecitabine, eribulin, gemcitabine, paclitaxel or nab-paclitaxel)	• Primary endpoint: PFS • Secondary endpoints: OS, DoR and ORR	• FPCD: Q4 2018 • LPCD: Q4 2020 • Data readout: Q1 2022 • Primary endpoint met
Phase III **DESTINY-Breast05** **NCT04622319** **Partnered (Daiichi Sankyo)**	High-risk HER2-positive with residual invasive breast cancer following neoadjuvant therapy	1600	• Randomised, open-label, parallel assignment • Arm 1: *Enhertu* • Arm 2: ado-trastuzumab emtansine	• Primary endpoint: IDFS • Secondary endpoints: DFS, OS, DRFI and BMFI	• FPCD: Q4 2020 • Data anticipated: H2 2025
Phase III **DESTINY-Breast06** **NCT04494425** **Partnered (Daiichi Sankyo)**	HER2-low and -ultralow, HR+ breast cancer with disease progression on endocrine therapy in the metastatic setting	866	• Randomised, open-label, parallel assignment • Arm 1: *Enhertu* • Arm 2: investigator's choice SoC chemotherapy (capecitabine, paclitaxel, nab-paclitaxel)	• Primary endpoint: PFS • Secondary endpoints: OS, DoR and ORR	• FPCD: Q3 2020 • LPCD: Q2 2023 • Data readout: Q2 2024 • Primary endpoint met
Phase III **DESTINY-Breast09** **NCT04784715** **Partnered (Daiichi Sankyo)**	HER2-positive, metastatic breast cancer with no prior therapy for advanced or metastatic disease	1157	• Randomised, parallel assignment • Arm 1: *Enhertu* + placebo • Arm 2: *Enhertu* + pertuzumab • Arm 3: SoC	• Primary endpoint: PFS • Secondary endpoints: OS, DoR and ORR	• FPCD: Q2 2021 • Data readout: Q2 2025 • Primary endpoint met for *Enhertu* + pertuzumab arm • Data anticipated for *Enhertu* monotherapy arm: H2 2026

As of 29 July 2025.
Appendix: Glossary.

Enhertu (trastuzumab deruxtecan, HER2 ADC)

Breast cancer

Trial	Population	Patients	Design	Endpoints	Status
Phase III **DESTINY-Breast11** **NCT05113251** **Partnered (Daiichi Sankyo)**	High-risk HER2-positive early non-metastatic breast cancer	927	• Randomised, open-label, parallel assignment • Arm 1: *Enhertu* • Arm 2: *Enhertu* followed by THP • Arm 3: doxorubicin and cyclophosphamide followed by THP	• Primary endpoint: pCR • Secondary endpoints: EFS, IDFS and OS	• FPCD: Q4 2021 • Data readout: Q2 2025 • Primary endpoint met
Phase Ib/II **DESTINY-Breast07** **NCT04538742** **Partnered (Daiichi Sankyo)**	HER2-positive metastatic breast cancer	245	• Randomised, open-label, sequential assignment • Arm 1: *Enhertu* • Arm 2: *Enhertu* + *Imfinzi* • Arm 3: *Enhertu* + pertuzumab • Arm 4: *Enhertu* + paclitaxel • Arm 5: *Enhertu* + *Imfinzi* + paclitaxel • Arm 6: *Enhertu* + tucatinib	• Primary endpoints: AE and SAE • Secondary endpoints: ORR, PFS, DoR and OS	• FPCD: Q1 2021 • Data anticipated: H2 2025
Phase Ib **DESTINY-Breast08** **NCT04556773** **Partnered (Daiichi Sankyo)**	HER2-low metastatic breast cancer	139	• Non-randomised, open-label parallel assignment • Arm 1: *Enhertu* + capecitabine • Arm 2: *Enhertu* + *Imfinzi* + paclitaxel • Arm 3: *Enhertu* + *Truqap* • Arm 4: *Enhertu* + anastrozole • Arm 5: *Enhertu* + *Faslodex*	• Primary endpoints: AE and SAE • Secondary endpoints: ORR, PFS, DoR and OS	• FPCD: Q1 2021 • LPCD: Q1 2023 • Data readout: Q3 2023

As of 29 July 2025.
Appendix: Glossary.

Enhertu (trastuzumab deruxtecan, HER2 ADC)

Gastric cancer

Trial	Population	Patients	Design	Endpoints	Status
Phase III **DESTINY-Gastric04** **NCT04704934** **Partnered (Daiichi Sankyo)**	HER2-positive gastric cancer or GEJ adenocarcinoma patients who have progressed on or after a trastuzumab-containing regimen and have not received any additional systemic therapy	490	• Open-label, randomised, parallel group assignment • Arm 1: *Enhertu* • Arm 2: SoC chemotherapy	• Primary endpoint: OS • Secondary endpoints: ORR, DoR, PFS, DcR and safety	• FPCD: Q2 2021 • Data readout: Q1 2025
Phase II **DESTINY-Gastric06** **NCT04989816** **Partnered (Daiichi Sankyo)**	HER2-positive gastric cancer or GEJ adenocarcinoma patients who have progressed on two prior treatment regimens	95	• Open-label, single group assignment • *Enhertu* • China only	• Primary endpoint: ORR • Secondary endpoints: PFS, ORR, DCR, OS, DoR and safety	• FPCD: Q3 2021 • LPCD: Q2 2024 • Data readout: Q3 2023
Phase Ib/II **DESTINY-Gastric03** **NCT04379596** **Partnered (Daiichi Sankyo)**	Metastatic or unresectable HER2+ GC, GEJ, & esophageal adenocarcinoma Part 1: ≥ 2L following trastuzumab containing therapy Part 2, 3 and 4: Previously untreated metastatic or unresectable GC Part 3 and 4: HER2 expressing (IHC 3+,2+,1+) (local assess	417	• Open-label, parallel assignment • Part 1: to determine recommended Phase II combination dose • 5 Arms combining *Enhertu* with SoC chemotherapies (5-FU, capecitabine, oxaliplatin) and/or *Imfinzi* • Part 2 and 3: to assess efficacy of the selected combinations • Arm 2A: standard chemotherapy • Arm 2B: *Enhertu* monotherapy • Arm 2C: *Enhertu* with chemotherapy • Arm 2D: *Enhertu* with chemotherapy and pembrolizumab • Arm 2E: *Enhertu* and pembrolizumab • Arm 2F: *Enhertu*, chemotherapy and pembrolizumab • Arm 3A (HER2+): *Enhertu*, chemotherapy and volrustomig • Arm 3B (HER2low): *Enhertu*, chemotherapy and volrustomig • Arm 4A (HER2+): *Enhertu*, chemotherapy and rilvegostomig • Arm 4B (HER2low): *Enhertu*, chemotherapy and rilvegostomig	• Primary endpoint (Part 1): safety, RP2D and ORR • Secondary endpoints: DoR, DCR, PFS, OS, PK parameters and presence of ADAs	• FPCD: Q2 2020 • Data anticipated: H2 2026
Phase III **DESTINY-Gastric05** **NCT06731478** **Partnered (Daiichi Sankyo)**	HER2+ 1L locally advanced or metastatic GC or GEJ adenocarcinoma	726	• Arm A (CPS ≥1): *Enhertu* + 5-FU or capecitabine + pembrolizumab • Arm B (CPS ≥1): *Enhertu* + 5-FU or capecitabine + cisplatin or oxaliplatin + pembrolizumab • Arm C (CPS <1): *Enhertu* + 5-FU or capecitabine • Arm D (CPS <1): ToGA	• Primary endpoint: PFS (BICR) in ITT • Secondary endpoints: OS, ORR, PFS (Inv.), DOR, safety and PRO	• FPCD: Q1 2025 • Data anticipated: >2026

As of 29 July 2025.
Appendix: Glossary.

Enhertu (trastuzumab deruxtecan, HER2 ADC)

Other cancers

Trial	Population	Patients	Design	Endpoints	Status
Phase III **DESTINY-Lung04** **NCT05048797** **Partnered (Daiichi Sankyo)**	HER2-mutated, unresectable, locally advanced/metastatic NSCLC	450	• Randomised, parallel group assignment • Arm 1: *Enhertu* • Arm 2: SoC (platinum, pemetrexed and pembrolizumab)	• Primary endpoint: PFS • Secondary endpoints: OS, CNS-PFS, PFS (INV), ORR, DoR, safety, PK parameters, ADA, PRO-tolerability and PRO- pulmonary symptoms	• FPCD: Q4 2021 • Data anticipated: H1 2026
Phase II **DESTINY-Lung02** **NCT04644237** **Partnered (Daiichi Sankyo)**	HER2-mutated, unresectable and/or metastatic NSCLC	152	• Randomised, parallel group assignment • Arm 1: *Enhertu* 6.4mg/kg • Arm 2: *Enhertu* 5.4mg/kg	• Primary endpoint: ORR • Secondary endpoints: DoR, DCR, PFS, OS and PK parameters	• FPCD: Q1 2021 • Data readout: Q1 2023 • Primary endpoint met
Phase II **DESTINY-PanTumor02** **NCT04482309** **Partnered (Daiichi Sankyo)**	HER2-expressing tumours	468	• Non-randomised, single group assignment • *Enhertu*	• Primary endpoint: ORR • Secondary endpoints: DoR, DCR, PFS and OS	• FPCD: Q4 2020 • Data readout: Q3 2023
Phase II **DESTINY-PanTumor01** **NCT04639219** **Partnered (Daiichi Sankyo)**	HER2-mutated tumours	102	• Non-randomised, single group assignment • *Enhertu*	• Primary endpoint: ORR • Secondary endpoints: DoR, DCR, PFS and PK parameters	• FPCD: Q1 2021 • LPCD: Q2 2022 • Data readout: Q2 2023
Phase II **DESTINY-CRC02** **NCT04744831** **Partnered (Daiichi Sankyo)**	HER2-overexpressing advanced or metastatic colorectal cancer	122	• Randomised, parallel group assignment • Arm 1: *Enhertu* 6.4mg/kg • Arm 2: *Enhertu* 5.4mg/kg	• Primary endpoint: ORR • Secondary endpoints: ORR, PFS, OS, DoR, DCR and PK parameters	• FPCD: Q1 2021 • Data readout: Q1 2023 • Primary endpoint met
Phase Ib **DESTINY-Lung03** **NCT04686305** **Partnered (Daiichi Sankyo)**	HER2-over expressing, unresectable and/or metastatic NSCLC Part 1: 2L/3L advanced Parts 2/3/4: 1L advanced	244	• Non-randomised, parallel group assignment • Part 1: to determine recommended combination dose • 3 Arms combine *Enhertu* with SoC chemotherapies (cisplatin, carboplatin or pemetrexed) and *Imfinzi*; Arm 1D: *Enhertu* monotherapy arm • Part 2: to assess efficacy of the selected combinations with chemotherapies (cisplatin, carboplatin or pemetrexed) and *Imfinzi* not initiated • Part 3 (2 arms): dose confirmation to assess safety and efficacy with volrustomig and volrustomig and chemotherapy (carboplatin) • Part 4 (2 arms): dose confirmation to assess safety and efficacy with rilvegostomig and rilvegostomig and chemotheraoy (carboplatin)	• Primary endpoint: safety and RP2D • Secondary endpoints: ORR, DoR, DCR, PFS, OS and PK parameters	• FPCD: Q4 2021 • Data anticipated: H2 2026

Enhertu (trastuzumab deruxtecan, HER2 ADC)

Other cancers

Trial	Population	Patients	Design	Endpoints	Status
Phase III **DESTINY-BTC01** **NCT06467357** **Partnered (Daiichi Sankyo)**	Advanced treatment-naïve HER2-expressing BTC	620	• Arm A: *Enhertu* + rilvegostomig • Arm B: *Enhertu* • Arm C: gemcitabine and cisplatin + *Imfinzi*	• Primary endpoint: OS • Secondary endpoint: OS (ITT), PFS (INV), ORR (ONV), DOR (INV) Safety, PRO	• FPCD: Q3 2024 • Data anticipated: >2026
Phase III **DESTINY-Endometrial01** **NCT06989112** **Partnered (Daiichi Sankyo)**	Stage III, Stage IV, or recurrent, histologically-confirmed endometrial cancer	600	• Open Label, Randomized, Global • Arm A: *Enhertu* + rilvegostomig • Arm B: *Enhertu* + pembrolizumab • Arm C: carboplatin/paclitaxel + pembrolizumab	• Primary: • • PFS (BICR) in ITT • • Secondary: • • OS • • PFS (Investigator) • • ORR	• FPCD: Q2 2025 • Data anticipated: >2026
Phase II **DESTINY-PanTumor03** **NCT06271837** **Partnered (Daiichi Sankyo)**	HER2 expressing tumours	125	• Non-randomised single group assignment • *Enhertu* • China only	• Primary endpoint: ORR • Secondary endpoints: DoR, DCR, PFS, OS, safety and tolerability, PK	• FPCD: Q3 2024 • Data anticipated: H2 2025
Phase II **DESTINY-Lung05** **NCT05246514** **Partnered (Daiichi Sankyo)**	HER2-mutant metastatic NSCLC who have disease progression on or after at least one-line of treatment	80	• Open-label, single-arm trial • China only	• Primary endpoint: ORR • Secondary endpoints: investigator and ICR assessed DCR, DoR and PFS, investigator assessed ORR, OS, ICR assessed NS-PFS, PK parameters, immunogenicity and safety	• FPCD: Q3 2022 • LPCD: Q1 2023 • Data readout: Q4 2023 • Primary endpoint met
Phase Ib **U106** **NCT04042701** **Partnered (Daiichi Sankyo)**	HER2-over expressing locally advanced/metastatic breast or NSCLC	115	• Non-randomised, parallel group assignment • *Enhertu* + pembrolizumab • Global trial – 2 countries	• Primary endpoints: DLT and ORR • Secondary endpoints: DoR, DCR, PFS, TTR and OS	• FPCD: Q2 2020 • Data anticipated: H2 2025

As of 29 July 2025.
Appendix: Glossary.

Imfinzi (PD-L1 mAb)

Gastrointestinal cancer

Trial	Population	Patients	Design	Endpoints	Status
Phase III **EMERALD-1** **NCT03778957**	Locoregional HCC	710	• Arm 1: TACE in combination with *Imfinzi* • Arm 2: TACE in combination with *Imfinzi* + bevacizumab • Arm 3: TACE in combination with placebo	• Primary endpoint: PFS (Arm 2 vs. Arm 3) • Secondary endpoints: PFS (Arm 1 vs. Arm 3) and OS	• FPCD: Q1 2019 • LPCD: Q3 2021 • Data readout: Q4 2023 • Primary endpoint met
Phase III **EMERALD-2** **NCT03847428**	HCC (adjuvant)	908	• Arm 1: *Imfinzi* + bevacizumab • Arm 2: *Imfinzi* + placebo • Arm 3: placebo + placebo	• Primary endpoint: RFS (Arm 1 vs. Arm 3) • Secondary endpoints: RFS (Arm 2 vs. Arm 3), OS and RFS at 24 months	• FPCD: Q2 2019 • LPCD: Q2 2022 • Data anticipated: H2 2026
Phase III **KUNLUN** **NCT04550260**	Locally advanced, unresectable ESCC	640	• Arm 1: *Imfinzi* + definitive CRT • Arm 2: placebo + definitive CRT	• Primary endpoint: PFS • Secondary endpoint: OS	• FPCD: Q4 2020 • LPCD: Q3 2023 • Data anticipated: H2 2026
Phase III **MATTERHORN** **NCT04592913**	Resectable GC/GEJC	900	• Arm 1: *Imfinzi* + FLOT • Arm 2: placebo + FLOT	• Primary endpoint: EFS • Secondary endpoints: OS (Arm 1 vs. Arm 2) and pCR (Arm 1 vs. Arm 2)	• FPCD: Q4 2020 • LPCD: Q3 2022 • Data readout: Q1 2025 • Primary endpoint met
Phase III **HIMALAYA** **NCT03298451**	1L HCC	1324	• Arm 1: *Imfinzi* + *Imjudo* • Arm 2: *Imfinzi* • Arm 3: sorafenib	• Primary endpoint: OS • Secondary endpoints: PFS, TTP and ORR	• FPCD: Q4 2017 • LPCD: Q4 2019 • Data readout: Q4 2021
Phase III **EMERALD-3** **NCT05301842**	Locoregional HCC	525	• Arm 1: TACE + T300 + D + lenvatanib • Arm 2: TACE + T300 + D • Arm 3: TACE	• Primary endpoint: PFS • Secondary endpoint: OS	• FPCD: Q2 2022 • Data anticipated: H1 2026

As of 29 July 2025.
Appendix: Glossary.

Imfinzi (PD-L1 mAb)

Lung cancer

Trial	Population	Patients	Design	Endpoints	Status
Phase III AEGEAN NCT03800134	Perioperative NSCLC patients, Stage II and III resected NSCLC (incl. EGFR/ALK-positive)	802	• Arm 1: *Imfinzi* + platinum-based chemotherapy • Arm 2: placebo + platinum-based chemotherapy	• Primary endpoints: pCR snd EFS • Secondary endpoints: mPR and DFS	• FPCD: Q1 2019 • Data readout: Q1 2023
Phase III ADJUVANT BR.31 NCT02273375 Partnered (CCTG)	Adjuvant NSCLC patients, Stage Ib (≥4cm) - Stage IIIa resected (incl. EGFR/ALK-positive)	1415	• Arm 1: *Imfinzi* mg/kg i.v. Q4W x 12 months • Arm 2: placebo • Global trial	• Primary endpoint: DFS • Secondary endpoint: OS	• FPCD: Q1 2015 • LPCD: Q1 2020 • Data readout: Q2 2024
Phase III PACIFIC-4 NCT03833154	Imfinzi with SBRT in unresected, Stage I/II NSCLC	630	• Arm 1: *Imfinzi* i.v. Q4W with definitive SBRT • Arm 2: placebo with definitive SBRT	• Primary endpoint: PFS • Secondary endpoint: OS	• FPCD: Q2 2019 • Data anticipated: >2026
Phase III PACIFIC-5 NCT03706690	Unresected, locally advanced NSCLC	407	• Arm 1: *Imfinzi* i.v. Q4W following chemotherapy/RT • Arm 2: placebo following chemotherapy/RT • Global trial (ex-US with China focus)	• Primary endpoint: PFS • Secondary endpoint: OS	• FPCD: Q1 2019 • LPCD: Q2 2022 • Data readout: Q3 2024
Phase III PACIFIC-8 NCT05211895 Partnered (Arcus Biosciences)	Unresected, locally advanced NSCLC	860	• Arm 1: *Imfinzi* + domvanalimab following chemotherapy/RT • Arm 2: *Imfinzi* + placebo following chemotherapy/RT	• Primary endpoint: PFS • Secondary endpoint: OS	• FPCD: Q1 2022 • Data anticipated: >2026
Phase III ADRIATIC NCT03703297	Limited-stage SCLC 1L following platinum-based concurrent chemoradiation therapy	730	• Arm 1: *Imfinzi* + *Imjudo* (4 doses) • Arm 2: *Imfinzi* • Arm 3: placebo	• Primary endpoints: PFS and OS	• FPCD: Q4 2018 • Data readout: Q2 2024 • Primary endpoint met
Phase III PACIFIC-9 NCT05221840 Partnered (Innate)	Patients with locally advanced (Stage III), unresectable NSCLC who have not progressed following platinum-based CRT	999	• Arm 1: *Imfinzi* + oleclumab • Arm 2: *Imfinzi* + monalizumab + placebo • Arm 3: *Imfinzi* + placebo	• Primary endoint: PFS • Secondary endpoints: OS, ORR, DoR, PFS2 and TFST	• FPCD: Q2 2022 • Data anticipated: H2 2026

As of 29 July 2025.
Appendix: Glossary.

Imfinzi (PD-L1 mAb)

Lung cancer

Trial	Population	Patients	Design	Endpoints	Status
Phase II **HUDSON** **NCT03334617**	NSCLC, patients who progressed on an anti-PD-1/PD-L1-containing therapy	531	• Open-label, biomarker-directed, multi-centre trial • Module 1: *Imfinzi* + *Lynparza* • Module 2: *Imfinzi* + danvatirsen • Module 3: *Imfinzi* + ceralasertib • Module 4: *Imfinzi* + vistusertib • Module 5: *Imfinzi* + oleclumab • Module 6: *Imfinzi* + *Enhertu* • Module 7: *Imfinzi* + cediranib • Module 8: ceralasertib • Module 9: *Imfinzi* + ceralasertib • Module 10: *Imfinzi* + ceralasertib • Module 11: ceralasertib	• Primary endpoint: ORR • Secondary endpoints: efficacy including OS, PFS, DCR, safety and tolerability and DoR	• FPCD: Q1 2018 • LPCD: Q3 2023 • Data readout: Q4 2024
Phase II **NeoCOAST-2** **NCT05061550**	Early-stage, resectable NSCLC (Stage II to Stage IIIA)	630	• Open-label trial • Arm 1: *Imfinzi* + oleclumab + platinum doublet chemotherapy • Arm 2: *Imfinzi* + monalizumab + platinum doublet chemotherapy • Arm 3: volrustomig + platinum doublet chemotherapy • Arm 4: *Datroway* + single agent platinum chemotherapy • Arm 5: AZD0171 + platinum doublet chemotherapy • Arm 6: rilvegostomig + platinum doublet chemotherapy • Arm 7: Dato-DXd + rilvegostomig + single agent platinum chemotherapy	• Primary endpoints: pCR and safety	• FPCD: Q2 2022 • Data anticipated: >2026

As of 29 July 2025.
Appendix: Glossary.

Imfinzi (PD-L1 mAb)

Other cancer

Trial	Population	Patients	Design	Endpoints	Status
Phase III **POTOMAC** **NCT03528694**	Non-muscle-invasive bladder cancer	1018	• Arm 1: BCG (induction + maintenance) • Arm 2: *Imfinzi* + BCG (induction only) • Arm 3: *Imfinzi* + BCG (induction + maintenance)	• Primary endpoint: DFS	• FPCD: Q2 2018 • LPCD: Q4 2020 • Data readout: Q2 2025
Phase III **NIAGARA** **NCT03732677**	Muscle-invasive bladder cancer eligible for cisplatin	1063	• Arm 1: *Imfinzi* in combination with gemcitabine + cisplatin, *Imfinzi* maintenance • Arm 2: gemcitabine + cisplatin	• Co-primary endpoints: pCR and EFS	• FPCD: Q4 2018 • LPCD: Q3 2021 • Data readout: Q2 2024
Phase III **SAMETA** **NCT05043090**	MET-driven, unresectable and locally advanced or metastatic papillary renal cell carcinoma	200	• Arm 1: *Orpathys* + *Imfinzi* • Arm 2: sunitinib • Arm 3: *Imfinzi* monotherapy	• Primary endpoint: PFS • Secondary endpoints: OS, ORR, DoR and DCR	• FPCD: Q4 2021 • Data anticipated: H1 2026
Phase III **NILE** **NCT03682068**	1L bladder cancer	1246	• Arm 1: *Imfinzi* + *Imjudo* + SoC • Arm 2: *Imfinzi* + SoC • Arm 3: SoC	• Primary endpoint: OS	• FPCD: Q4 2018 • LPCD: Q2 2021 • Data anticipated: H1 2026
Phase III **VOLGA** **NCT04960709**	Muscle-invasive bladder cancer ineligible to cisplatin	712	• Arm 1: *Imfinzi* + *Imjudo* + enfortumab vedotin • Arm 2: *Imfinzi* + enfortumab vedotin • Arm 3: SoC cystectomy	• Primary endpoints: safety, EFS and pCR • Secondary endpoint: OS	• FPCD: Q4 2021 • LPCD: Q1 2025 • Data anticipated: H2 2025
Phase II **BEGONIA** **NCT03742102**	1L mTNBC	243	• Arm 1: *Imfinzi* + paclitaxel • Arm 2: *Imfinzi* + paclitaxel + *Truqap* • Arm 5: *Imfinzi* + paclitaxel + oleclumab • Arm 6: *Imfinzi* + *Enhertu* • Arm 7: *Imfinzi* + *Datroway* • Arm 8: *Imfinzi* + *Datroway* (PD-L1-high) • Global trial	• Primary endpoints: safety and tolerability • Secondary endpoints: ORR, PFS, DoR, OS, PK and ADA	• FPCD: Q1 2019 • Data readout: Q2 2025

As of 29 July 2025.
Appendix: Glossary.

Lynparza (PARP inhibitor)

Imfinzi combinations

Trial	Population	Patients	Design	Endpoints	Status
Phase III **DUO-O** **NCT03737643**	1L advanced ovarian cancer	1407	• Non-tBRCAm (tumour BRCA) patients • Arm 1: chemotherapy + bevacizumab + *Imfinzi* placebo followed by bevacizumab + *Imfinzi* placebo + *Lynparza* placebo • Arm 2: chemotherapy + bevacizumab + *Imfinzi* followed by bevacizumab + *Imfinzi* + *Lynparza* placebo • Arm 3: chemotherapy + bevacizumab + *Imfinzi* followed by bevacizumab + *Imfinzi* + *Lynparza* • tBRCAm patients • chemotherapy + bevacizumab (optional) + *Imfinzi* followed by bevacizumab (optional) + *Imfinzi* + *Lynparza* • Global trial	• Primary endpoint: PFS • Secondary endpoints: OS and PFS2	• FPCD: Q1 2019 • LPCD: Q2 2023 • Data readout: Q2 2023 • Primary endpoint met
Phase III **DUO-E** **NCT04269200**	1L advanced and recurrent endometrial cancer	805	• Arm 1: chemotherapy + *Imfinzi* placebo followed by *Imfinzi* placebo + *Lynparza* placebo • Arm 2: chemotherapy + *Imfinzi* followed by *Imfinzi* + *Lynparza* placebo • Arm 3: chemotherapy + *Imfinzi* followed by *Imfinzi* + *Lynparza* • Global trial	• Primary endpoint: PFS • Secondary endpoints: OS, PFS2, ORR and DoR	• FPCD: Q2 2020 • LPCD: Q2 2023 • Data readout: Q2 2023 • Primary endpoint met

As of 29 July 2025.
Appendix: Glossary.

Lynparza (PARP inhibitor)

Other cancer

Trial	Population	Patients	Design	Endpoints	Status
Phase III **MONO-OLA1** **NCT04884360**	BRCAwt advanced ovarian cancer, 1L maintenance	366	• Arm 1: *Lynparza* BID 24-month duration • Arm 2: placebo BID 24-month duration • Global trial – 12 countries	• Primary endpoints: PFS (BRCAwt HRD-positive) and PFS (BRCAwt) • Secondary endpoints: OS, TFST and PFS2	• FPCD: Q3 2021 • LPCD: Q1 2024 • Data anticipated: H2 2025
Phase III **PROpel** **NCT03732820**	1L metastatic castration-resistant prostate cancer	906	• Arm 1: *Lynparza* + abiraterone • Arm 2: placebo + abiraterone • Global trial (including China)	• Primary endpoint: rPFS • Secondary endpoint: OS	• FPCD: Q4 2018 • LPCD: Q3 2022 • Data readout: Q3 2021 • Primary endpoint met

As of 29 July 2025.
Appendix: Glossary.

Orpathys (savolitinib, MET inhibitor)

NSCLC and other cancers

Trial	Population	Patients	Design	Endpoints	Status
Phase III **NCT04923945** **Partnered (HUTCHMED)**	Locally advanced or metastatic NSCLC patients with MET exon 14 mutations without EGFR, ALK and ROS1 mutations progressing on platinum chemotherapy and are treatment naïve to c-MET therapy or did not receive prior drug therapy for advanced tumours	163	• Single-arm trial • *Orpathys*	• Primary endpoint: ORR	• FPCD: Q3 2021 • LPCD: Q2 2023 • Data readout: Q4 2024
Phase II **NCT04923932** **Partnered (HUTCHMED)**	Locally advanced or metastatic gastric cancer and esophagogastric junction adenocarcinoma patients with MET gene amplifications	75	• Single-arm, multi-cohort, multi-centre, open-label trial • *Orpathys*	• Primary endpoint: ORR • Secondary endpoints: PFS and safety	• FPCD: Q3 2021 • Data anticipated: H2 2025

As of 29 July 2025.
Appendix: Glossary.

Tagrisso (highly-selective, irreversible EGFR inhibitor)

NSCLC

Trial	Population	Patients	Design	Endpoints	Status
Phase III **LAURA** **NCT03521154**	Maintenance therapy in patients with locally advanced, unresectable EGFRm Stage III NSCLC whose disease has not progressed following platinum-based chemoradiation therapy	216	• Arm 1: *Tagrisso* • Arm 2: placebo	• Primary endpoint: PFS (BICR) • Secondary endpoints: CNS PFS, OS, DoR, ORR and DCR	• FPCD: Q4 2018 • LPCD: Q3 2022 • Data readout: Q1 2024 • Primary endpoint met
Phase III **ADAURA2** **NCT05120349**	Adjuvant EGFRm NSCLC Stage IA2 to IA3 following complete tumour resection	380	• Arm 1: *Tagrisso* • Arm 2: placebo	• Primary endpoint: DFS • Secondary endpoints: DFS rate, OS, OS rate and QoL	• FPCD: Q2 2022 • LPCD: Q4 2024 • Data anticipated: >2026

As of 29 July 2025.
Appendix: Glossary.

Tagrisso (highly-selective, irreversible EGFR inhibitor)

NSCLC, combinations

Trial	Population	Patients	Design	Endpoints	Status
Phase III **NeoADAURA** **NCT04351555**	Neoadjuvant EGFRm NSCLC	351	• Arm 1: placebo + pemetrexed/carboplatin or pemetrexed/cisplatin • Arm 2: *Tagrisso* + pemetrexed/carboplatin or pemetrexed/cisplatin • Arm 3: *Tagrisso*	• Primary endpoint: mPR • Secondary endpoints: cPR, EFS, DFS and OS	• FPCD: Q1 2021 • LPCD: Q4 2023 • Data readout: Q4 2024 • Primary endpoint met
Phase III **SAFFRON** **NCT05261399** **Partnered (HUTCHMED)**	EGFRm, MET-overexpressed and/or amplified, locally advanced or metastatic NSCLC patients who have progressed on first- or second-line treatment with Tagrisso	324	• Arm 1: *Tagrisso* + *Orpathys* • Arm2: pemetrexed with either cisplatin or carboplatin	• Primary enpoint: PFS • Secondary endpoints: OS, ORR, PK, DCR and DoR	• FPCD: Q3 2022 • Data anticipated: H1 2026
Phase III **SANOVO** **NCT05009836** **Partnered (HUTCHMED)**	1L EGFRm, MET+ locally advanced or metastatic NSCLC	320	• Arm 1: *Tagrisso* + *Orpathys* • Arm 2: *Tagrisso* + placebo	• Primary endpoint: PFS	• FPCD: Q3 2021 • Data anticipated: H2 2026
Phase III **SACHI** **NCT05015608** **Partnered (HUTCHMED)**	Locally advanced or metastatic NSCLC with MET amplification after failure of the first-line EGFR inhibitor therapy	250	• Arm 1: *Tagrisso* + *Orpathys* • Arm 2: pemetrexed + platinum • China only	• Primary endpoint: PFS	• FPCD: Q3 2021 • Data readout: Q3 2024 • primary endpoint met
Phase II **SAVANNAH** **NCT03778229** **Partnered (HUTCHMED)**	EGFRm/MET+, locally advanced or metastatic NSCLC who have progressed following treatment with Tagrisso	360	• Protocol v1-6: single-arm, open-label trial • Protocol v7: randomised, double-blind trial • Arm 1: *Tagrisso* + *Orpathys* • Arm 2: placebo + *Orpathys*	• Primary endpoint: ORR • Secondary endpoints: PFS, DoR and OS	• FPCD: Q1 2019 • LPCD: Q1 2024 • Data readout: Q3 2024 • Clinically meaningful ORR
Phase II **ORCHARD** **NCT03944772**	Advanced EGFRm NSCLC patients who have progressed on first-line Tagrisso treatment	250	• Modular design platform trial: • Module 1: *Tagrisso* + *Orpathys* (cMET) • Module 2: *Tagrisso* + gefitinib (EGFRm) • Module 3: *Tagrisso* + necitumumab (EGFRm) • Module 4: carboplatin + pemetrexed + *Imfinzi* • Module 5: *Tagrisso* + alectinib (ALK) • Module 6: *Tagrisso* + selpercatinib (RET fusion) • Module 7: *Imfinzi* + etoposide + carboplatin or cisplatin • Module 8: *Tagrisso* + pemetrexed + carboplatin or cisplatin • Module 9: *Tagrisso* + *Koselugo* • Module 10: *Tagrisso* + datopotamab deruxtecan • No intervention: observational cohort •	• Primary endpoint: ORR • Secondary endpoints: PFS, DoR, OS, safety and tolerability	• FPCD: Q3 2019 • LPCD: Q4 2023 • Data anticipated: H2 2025

As of 29 July 2025.
Appendix: Glossary.

Truqap (capivasertib, AKT inhibitor)

Breast cancer and prostate cancer

Trial	Population	Patients	Design	Endpoints	Status
Phase III **CAPItello-291** **NCT04305496**	2L+ AI-resistant locally advanced (inoperable) or metastatic HR+ HER2-negative breast cancer	834	• Double-blind, randomised, comparative trial • Arm 1: *Truqap* + *Faslodex* • Arm 2: placebo + *Faslodex*	• Primary endpoint: PFS	• FPCD: Q2 2020 • LPCD: Q4 2021 • Data readout: Q4 2022 • Both primary endpoints met
Phase III **CAPItello-281** **NCT04493853**	De novo PTEN deficient metastatic hormone sensitive prostate cancer	1012	• Double-blind, randomised, comparative trial • Arm 1: *Truqap* + abiraterone • Arm 2: placebo + abiraterone	• Primary endpoint: rPFS	• FPCD: Q3 2020 • LPCD: Q1 2024 • Data readout: Q4 2024 • Primary endpoint met
Phase Ib/III **CAPItello-292** **NCT04862663**	1L triplet in early relapse/endocrine-resistant locally advanced (inoperable) or metastatic HR+/HER2-negative breast cancer	793	• Double-blind, randomised, comparative trial • Arm 1: *Truqap* + palbociclib + *Faslodex* • Arm 2: placebo + palbociclib + *Faslodex*	• Primary endpoint: PFS	• FPCD: Q2 2021 • Data anticipated: >2026

As of 29 July 2025.
Appendix: Glossary.

camizestrant (next-generation oral SERD)

Breast cancer

Trial	Population	Patients	Design	Endpoints	Status
Phase III SERENA-4 NCT04711252	HR+ HER2-negative advanced breast cancer	1370	• Randomised, double-blind, comparative trial • Arm 1: camizestrant + palbociclib • Arm 2: anastrazole + palbociclib	• Primary endpoint: PFS • Secondary endpoints: OS and PFS2	• FPCD: Q1 2021 • LPCD: Q4 2023 • Data anticipated: H2 2026
Phase III SERENA-6 NCT04964934	HR+ HER2-negative advanced breast cancer	312	• Randomised, double-blind, comparator trial • Arm 1: camizestrant + palbociclib or abemaciclib or ribociclib • Arm 2: anastrazole or letrozole + palbociclib or abemaciclib or ribociclib	• Primary endpoint: PFS • Secondary endpoint: OS and PFS2	• FPCD: Q3 2021 • LPCD: Q3 2024 • Data readout: Q1 2025 • Primary endpoint met
Phase III CAMBRIA-1 NCT05774951	ER+/HER2-negative early breast cancer patients who completed definitive locoregional therapy and standard adjuvant ET for at least 2 years and up to 5 years	4300	• Arm 1: continue standard ET of investigator's choice • Arm 2: camizestrant	• Primary endpoint: IBCFS • Secondary endpoints: IDFS, DRFS and OS	• FPCD: Q1 2023 • Data anticipated: >2026
Phase III CAMBRIA-2 NCT05952557	ER+/HER2-negative early breast cancer with intermediate-high or high risk of recurrence that has completed definitive locoregional therapy and have no evidence of disease	5500	• Arm A: standard endocrine therapy of investigator's choice (aromatase inhibitors [exemestane, letrozole, anastrozole] or tamoxifen) ± abemaciclib • Arm B: camizestrant ± abemaciclib	• Primary endpoint: IBCFS • Secondary endpoints: IDFS, DRFS and OS	• FPCD: Q4 2023 • Data anticipated: >2026
Phase II SERENA-2 NCT04214288	HR+ advanced breast cancer	240	• Randomised, open-label, parallel-group, multi-centre trial • Arm 1: camizestrant (75mg) • Arm 2: camizestrant (150mg) • Arm 3: camizestrant (300mg) • Arm 4: *Faslodex*	• Primary endpoint: PFS	• FPCD: Q2 2020 • LPCD: Q3 2021 • Data readout: Q4 2022 • Primary endpoint met at 75mg and 150mg doses

As of 29 July 2025.
Appendix: Glossary.

camizestrant (next-generation oral SERD)

Breast cancer

Trial	Population	Patients	Design	Endpoints	Status
Phase I **NCT04541433**	HR+ HER2-negative advanced breast cancer	18	• Open-label trial • camizestrant • Japan only	• Primary endpoints: safety and tolerability • Secondary endpoint: PK parameters	• FPCD: Q4 2020 • LPCD: Q1 2022 • Data readout: Q1 2023
Phase I **SERENA-1** **NCT03616587**	HR+ HER2-negative advanced breast cancer	396	• Escalation phase: open-label multi-centre trial • Cohort 1: camizestrant • Cohort 2: camizestrant + palbociclib, everolimus, abemeciclib (+/- anastrozole), *Truqap*, ribociclib (+/- anastrozole) or anastrozole • Expansion phase: randomised expansion cohort(s) • Cohort 1: camizestrant • Cohort 2: camizestrant + palbociclib, everolimus, abemeciclib (+/- anastrozole), *Truqap*, ribociclib (+/- anastrozole) or anastrozole	• Primary endpoints: safety and tolerability • Secondary endpoints: PK parameters and anti-tumour activity	• FPCD: Q4 2018 • LPCD: Q1 2024 • Data readout: Q1 2025
Phase I **NCT04818632**	HR+ HER2-negative metastatic breast cancer in Chinese patients	30	• Dose escalation: camizestrant • Dose expansion: • Cohort 1: camizestrant • Cohort 2: camizestrant + palbociclib • Cohort 3: camizestrant + everolimus • China only	• Primary endpoints: safety, tolerability and PK parameters • Secondary endpoint: anti-tumour activity	• FPCD: Q1 2021 • LPCD: Q1 2023 • Data readout: Q4 2023

As of 29 July 2025.
Appendix: Glossary.

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Appendix: Glossary.

ceralasertib (AZD6738, ATR inhibitor)

Multiple cancers

Trial	Population	Patients	Design	Endpoints	Status
Phase III **LATIFY** **NCT05450692**	Post-IO NSCLC	594	• Double-arm randomised • Arm 1: ceralasertib + *Imfinzi* • Arm 2: docetaxel	• Primary endpoint: OS • Secondary endpoints: PFS, ORR, DoR, TTR, DCR, PFS2 and TTD	• FPCD: Q4 2022 • Data anticipated: H2 2025
Phase I/II **NCT02264678**	Solid tumours	357	• Module 1: ceralasertib + carboplatin • Module 2: ceralasertib dose escalation, ceralasertib + *Lynparza* • Module 3: ceralasertib + *Imfinzi* • Module 4: ceralasertib monotherapy + *Lynparza* + *Imfinzi* (food effect/QT) • Module 5: ceralasertib + saruparib • Global trial – North America, Europe and South Korea	• Primary endpoints: safety and tolerability, efficacy and PK parameters	• FPCD: Q4 2014 • Data anticipated: H2 2025

As of 29 July 2025.
Appendix: Glossary.

rilvegostomig (PD-1/TIGIT bispecific mAb)

Lung cancer

Trial	Population	Patients	Design	Endpoints	Status
Phase III ARTEMIDE-Lung03 NCT06627647 Partnered (Compugen)	Non-squamous NSCLC 1L patients whose tumours express PD-L1 (TC ≥1%)	878	• Randomised, double-blind, multi-centre trial • Arm 1: rilvegostomig + platinum-based doublet chemotherapy followed by rilvegostomig monotherapy + pemetrexed in maintenance • Arm 2: pembrolizumab + platinum-based doublet chemotherapy followed by pembrolizumab monotherapy + pemetrexed in maintenance	• Primary endpoints: PFS and OS • Secondary endpoints: OS, ORR and DoR	• FPCD: Q4 2024 • Data anticipated: >2026
Phase III ARTEMIDE-Lung02 NCT06692738 Partnered (Compugen)	squamous NSCLC 1L patients whose tumours express PD-L1 (TC >/=1%)	880	• randomized, double-blind, multicenter, • Arm 1: Rilvegostomig + platinum-based doublet chemotherapy followed by rilvegostomig maintenance. • Arm 2: pembrolizumab + platinum-based doublet chemotherapy followed by pembrolizumab maintenance.	• Primary endpoints: PFS, OS • Secondary endpoint: OS, ORR, DoR	• FPCD: Q4 2024 • Data anticipated: >2026
Phase III ARTEMIDE-Lung04 NCT06868277 Partnered (Compugen)	NSCLC 1L patients whose tumours express PD-L1 (TC >/= 50%)	830	• randomised, double-blind, multicentre • Arm A: rilvegostomig • Arm B: pembrolizumab	• Primary endpoints: PFS, OS • Secondary endpoints: ORR, DoR	• Data anticipated: >2026 • Initiating
Phase I/II ARTEMIDE-01 NCT04995523 Partnered (Compugen)	NSCLC	210	• Open-label, dose escalation and dose expansion trial • Part A: dose escalation in CPI-experienced NSCLC patients with rilvegostomig i.v. monotherapy • Part B: dose expansion in CPI-experienced NSCLC patients with rilvegostomig i.v. monotherapy • Part C: dose expansion in CPI-naive NSCLC patients with rilvegostomig i.v. monotherapy • Part D: randomised dose expansion in CPI-naive NSCLC patients with rilvegostomig i.v. monotherapy • Part E: dose expansion in CPI-naive stage IV squamous NSCLC patients with rilvegostomig i.v. monotherapy • Global trial	• Primary endpoints (Part A): safety, RP2D and MTD • Primary endpoints (Part B): safety and efficacy (ORR) • Primary endpoints (Part C): safety and efficacy (ORR) • Primary endpoints (Part D): safety and efficacy (ORR) • Primary endpoints (Part E): safety and efficacy (ORR) • Secondary endpoints: PK parameters, PD (receptor occupancy), efficacy (DCR, DoR, DRR, PFS)	• FPCD: Q4 2021 • Data anticipated: H2 2026

As of 29 July 2025.
Appendix: Glossary.

rilvegostomig (PD-1/TIGIT bispecific mAb)

Gastrointestinal cancers

Trial	Population	Patients	Design	Endpoints	Status
Phase III **ARTEMIDE-Biliary01** **NCT06109779** **Partnered (Compugen)**	adjuvant BTC with curative intent	750	• Randomised, double-blind, placebo-controlled, multicenter • Arm 1: rilvegostomig + investigator's choice of chemotherapy (capecitabine, S-1 (tegafur/gimeracil/oteracil) or gemcitabine/cisplatin) • Arm 2: placebo + investigator's choice of chemotherapy (capecitabine, S-1 (tegafur/gimeracil/oteracil) or gemcitabine/cisplatin)	• Primary endpoint: RFS • Secondary endpoint: OS	• FPCD: Q4 2023 • Data anticipated: >2026
Phase III **ARTEMIDE-HCC01** **NCT06921785** **Partnered (Compugen)**	Patients with advanced hepatocellular cancer who are not amenable to curative therapy or locoregional therapy	1220	• randomised, open-label, sponsor-blinded, 3-arm, multicentre, global • Arm A: Tremelimumab, rilvegostomig and bevacizumab • Arm B: Rilvegostomig and bevacizumab • Arm C: Atezolizumab and bevacizumab	• Primary endpoint: OS • Secondary endpoints: PFS, ORR, DoR	• FPCD: Q2 2022 • Data anticipated: >2026
Phase III **ARTEMIDE-Gastric01** **NCT06764875** **Partnered (Compugen)**	HER2-positive locally advanced or metastatic gastric or GEJ adenocarcinoma participants whose tumors express PD L1 CPS ≥ 1.	840	• Randomised, multicentre • Arm A: rilvegostomig in combination with fluoropyrimidine and T-DXd • Arm B: trastuzumab in combination with chemotherapy and pembrolizumab • Arm C: trastuzumab in combination with chemotherapy and rilvegostomig	• Primary endpoints: PFS, OS • Secondary endpoints: ORR, DoR	• FPCD: Q1 2025 • Data anticipated: >2026

As of 29 July 2025.
Appendix: Glossary.

rilvegostomig (PD-1/TIGIT bispecific mAb)

Gastrointestinal cancers

Trial	Population	Patients	Design	Endpoints	Status
Phase IIb **GEMINI-Gastric** **NCT05702229** **Partnered (Compugen)**	Gastric cancer	240	• Open-label gastric platform trial • Sub-study 1: volrustomig combined with XELOX or FOLFOX • Sub-study 2: rilvegostomig combined with XELOX or FOLFOX • Sub-study 3: AZD0901 combined with volrustomig plus fluorouracil or capecitabine • Sub-study 4: AZD0901 combined with rilvegostomig plus fluorouracil or capecitabine • Sub-study 5: AZD7789 combined with XELOX or FOLFOX • Sub-study 6: AZD0901 combined with AZD7789 plus fluorouracil or capecitabine	• Primary endpoints: safety and efficacy (ORR and PFS6) • Secondary endpoints: DoR, OS, PK, ADA and safety	• FPCD: Q1 2023 • Data anticipated: H2 2026
Phase IIb **GEMINI-PeriOp Gastric** **NCT07069712** **Partnered (Compugen)**	Gastroesophageal Adenocarcinoma	150	• Open-label platform study • Substudy 1: AZD0901 plus rilvegostomig and 5-FU or capecitabine • Substudy 2: T-DXd plus rilvegostomig and 5-FU or capecitabine • Substudy 3: Rilvegostomig plus FLOT chemotherapy	• Primary endpoints: safety, pCR rate	• FPCD: Q3 2025 • Data anticipated: H2 2026
Phase IIb **GEMINI-Hepatobiliary** **NCT05775159** **Partnered (Compugen)**	HCC, BTC	260	• Open-label hepatobiliary platform trial • HCC sub-study: • Cohort 1A: volrustomig monotherapy • Cohort 1B: volrustomig combination with bevacizumab • Cohort 1C: volrustomig combination with lenvatinib • Cohort 1D: volrustomig combination with rilvegostomig and bevacizumab • Cohort 1E: rilvegostomig combination with bevacizumab • BTC sub-study: • Cohort 2A: rilvegostomig combination with gemcitabine and cisplatin • Cohort 2B: volrustomig combination with gemcitabine and cisplatin	• Primary endpoints (HCC sub-study): safety and efficacy (ORR) • Primary endpoints (BTC sub-study): safety and efficacy (PFS6) • Secondary endpoints: DoR, OS, PK and ADA	• FPCD: Q2 2023 • Data anticipated: H2 2026

As of 29 July 2025.
Appendix: Glossary.

saruparib (AZD5305, PARP1 inhibitor)

Solid tumours

Trial	Population	Patients	Design	Endpoints	Status
Phase III **EvoPAR-Prostate01** **NCT06120491** -	HRRm and non-HRRm mCSPC	1800	• Randomised, placebo-controlled trial • Arm 1: saruparib + physician's choice NHA (abiraterone, darolutamide or enzalutamide) • Arm 2: placebo + physician's choice NHA (abiraterone, darolutamide or enzalutamide)	• Primary endpoint: rPFS • Secondary endpoints: OS and PFS2	• FPCD: Q4 2023 • Data anticipated: >2026
Phase III **EvoPAR-Breast01** **NCT06380751**	BRCA1, BRCA2, or PALB2m, HR-positive, HER2-negative advanced breast cancer	500	• Randomised, open-label trial • Arm 1: saruparib (AZD5305) + camizestrant • Arm 2: physician's choice CDK4/6i + physician's choice ET • Arm 3: physician's choice CDK4/6i + camizestrant	• Primary endpoint: PFS (BICR) • Secondary endpoints: PFS2 and OS	• FPCD: Q3 2024 • Data anticipated: >2026

As of 29 July 2025.
Appendix: Glossary.

saruparib (AZD5305, PARP1 inhibitor)

Solid tumours

Trial	Population	Patients	Design	Endpoints	Status
Phase I/IIa **PETRA** **NCT04644068**	Advanced solid tumours	804	• Modular, open-label, multi-centre dose escalation and expansion trial • Module 1: saruparib • Module 2: saruparib + paclitaxel • Module 3: saruparib + carboplatin +/- paclitaxel • Module 4: saruparib + *Enhertu* • Module 5: saruparib + *Datroway* • Module 6: saruparib + camizestrant	• Primary endpoints: safety, tolerability and PK parameters • Secondary endpoint: efficacy	• FPCD: Q4 2020 • Data anticipated: >2026
Phase I/IIa **PETRANHA** **NCT05367440**	Metastatic prostate cancer	190	• Multi-arm, open-label, non-randomised, multi-centre trial of saruparib in combination with new hormonal agents in patients with metastatic prostate cancer • Arm 1: saruparib + enzalutamide • Arm 2: saruparib + abiraterone acetate • Arm 3: saruparib + darolutamide • Arm 4: saruparib + apalutamide	• Primary endpoints: safety and tolerability • Secondary endpoints: PK parameters and efficacy	• FPCD: Q2 2022 • Data anticipated: >2026
Phase I **NCT05573724**	Locally advanced, unresectable or metastatic solid tumours	16	• Part A: to assess the effect of multiple doses of itraconazole on the single-dose PK parameters of saruparib which will last up to 13 days and follows a non-randomised, open-label, 2 intervention design • Part B: option to continue with saruparib monotherapy after completing Part A and whilst obtaining clinical benefit	• Primary endpoint: PK parameters • Secondary endpoints: safety and tolerability	• FPCD: Q4 2022 • LPCD: Q2 2023 • Data readout: Q4 2023 • Primary endpoint met
Phase I **ASCERTAIN** **NCT05938270**	Newly diagnosed prostate cancer	120	• Open-label, randomised, multi-centre trial	• Primary endpoint: to assess the effects of treatment on γH2AX change • Secondary endpoints: safety and tolerability, impact on surgical feasibility and change in Ki67	• FPCD: Q3 2023 • Data anticipated: H1 2026

As of 29 July 2025.
Appendix: Glossary.

sonesitatug vedotin (AZD0901, CLDN18.2 MMAE ADC)

Solid tumours

Trial	Population	Patients	Design	Endpoints	Status
Phase III **CLARITY- Gastric 01** **NCT06346392**	2L+ advanced or metastatic gastric or GEJ adenocarcinoma expressing CLDN18.2	572	• Multi-centre, open-label, sponsor-blinded, randomised trial • Arm 1: AZD0901 dose level 1 via i.v. infusion treatment • Arm 2: AZD0901 dose level 2 via i.v. infusion treatment • Arm 3: investigator's choice chemotherapies	• Primary endpoints: PFS and OS • Secondary endpoints: OS, PFS for 3L+, ORR, ORR for 3L+, DoR, MMAE, safety and tolerability, PK parameters and prevalence of ADAs	• FPCD: Q2 2024 • Data anticipated: H1 2026
Phase II **CLARITY-PanTumour01** **NCT06219941**	Locally advanced unresectable or metastatic solid tumours expressing CLDN18.2	190	• Open-label, multi-centre trial of AZD0901 administered via i.v. • Sub-study 1: AZD0901 monotherapy (Gastric Cancer) • Sub-study 2: AZD0901 and anti-cancer agents (Pancreatic Cancer) • Sub-study 3: AZD0901 monotherapy (Biliary Tract Cancer)	• Primary endpoints: AEs, SAEs and ORR • Secondary endpoints: OS, PFS, DoR, DCR, PK parameters and prevalence of ADAs	• FPCD: Q1 2024 • Data anticipated: >2026

As of 29 July 2025.
Appendix: Glossary.

surovatamig (AZD0486, CD19/CD3 next-generation bispecific T-cell engager)

Haematologic malignancies

Trial	Population	Patients	Design	Endpoints	Status
Phase III **SOUNDTRACK-F1** **NCT06549595**	Previously untreated follicular lymphoma	1015	• Multi-centre, randomised, open-label trial • Arm 1: rituximab + AZD0486 followed by observation • Arm 2: rituximab + AZD0486 followed by maintenance AZD0486 • Arm 3: Investigator's choice of RCHOP/RCVP/BR followed by standard of care maintenance or observation	• Primary endpoint: PFS • Secondary endpoint: CR	• FPCD: Q3 2024 • Data anticipated: >2026
Phase II **SOUNDTRACK-B** **NCT06526793**	B-cell non-Hodgkin lymphoma, follicular lymphoma and diffuse large B-cell lymphoma	240	• Multi-centre, single-arm, open-label trial • Module 1 Follicular Lymphoma • Module 2 Diffuse large B-cell lymphoma	• Primary endpoint: ORR • Secondary endpoints: DoR, CR and PFS	• FPCD: Q4 2024 • Data anticipated: >2026
Phase I/II **SYRUS** **NCT06137118**	R/R B-ALL	120	• Multi-centre, open-label, single-arm dose escalation and dose optimisation trial	• Primary endpoints: DLT, safety and ORR • Secondary endpoints: ORR, DoR, CR rate at any time during trial, EFS, OS, subsequent alloSCT, CR MRD-negative rate, PK parameters and ADA	• FPCD: Q1 2024 • Data anticipated: H2 2025
Phase I/II **SOUNDTRACK-E** **NCT06564038**	Mature B-cell malignancies (chronic lymphocytic leukaemia/small lymphocytic leukaemia, mantle-cell lymphoma, and large B-cell lymphoma)	180	• Multi-centre, open-label trial • Sub-study 1 (RR CLL/SLL): AZD0486 in IV or SC • Sub-study 2 (RR MCL): AZD0486 in IV or SC • Sub-study 3 (RR LBCL): AZD0486 IV + R-CHOP	• Primary endpoints: DLT and safety • Secondary endpoints: ORR, CR rate, DoR, Cmax, AUC, Cmin, Tmax, Ctrough, half-life of AZD0486, clearance of AZD0486 and ADA	• FPCD: Q1 2025 • Data anticipated: H2 2026
Phase I **NCT04594642**	R/R B-cell non-Hodgkin lymphoma	317	• Multi-centre, open-label, dose escalation and dose expansion trial	• Primary endpoints: safety and tolerability, MTD and/or RP2D and PK parameters • Secondary endpoints: clinical activity of AZD0486 monotherapy and ADA titers for AZD0486 monotherapy	• FPCD: Q1 2021 • Data anticipated: H2 2026

As of 29 July 2025.
Appendix: Glossary.

volrustomig (PD-1/CTLA-4 bispecific mAb)

Solid tumours

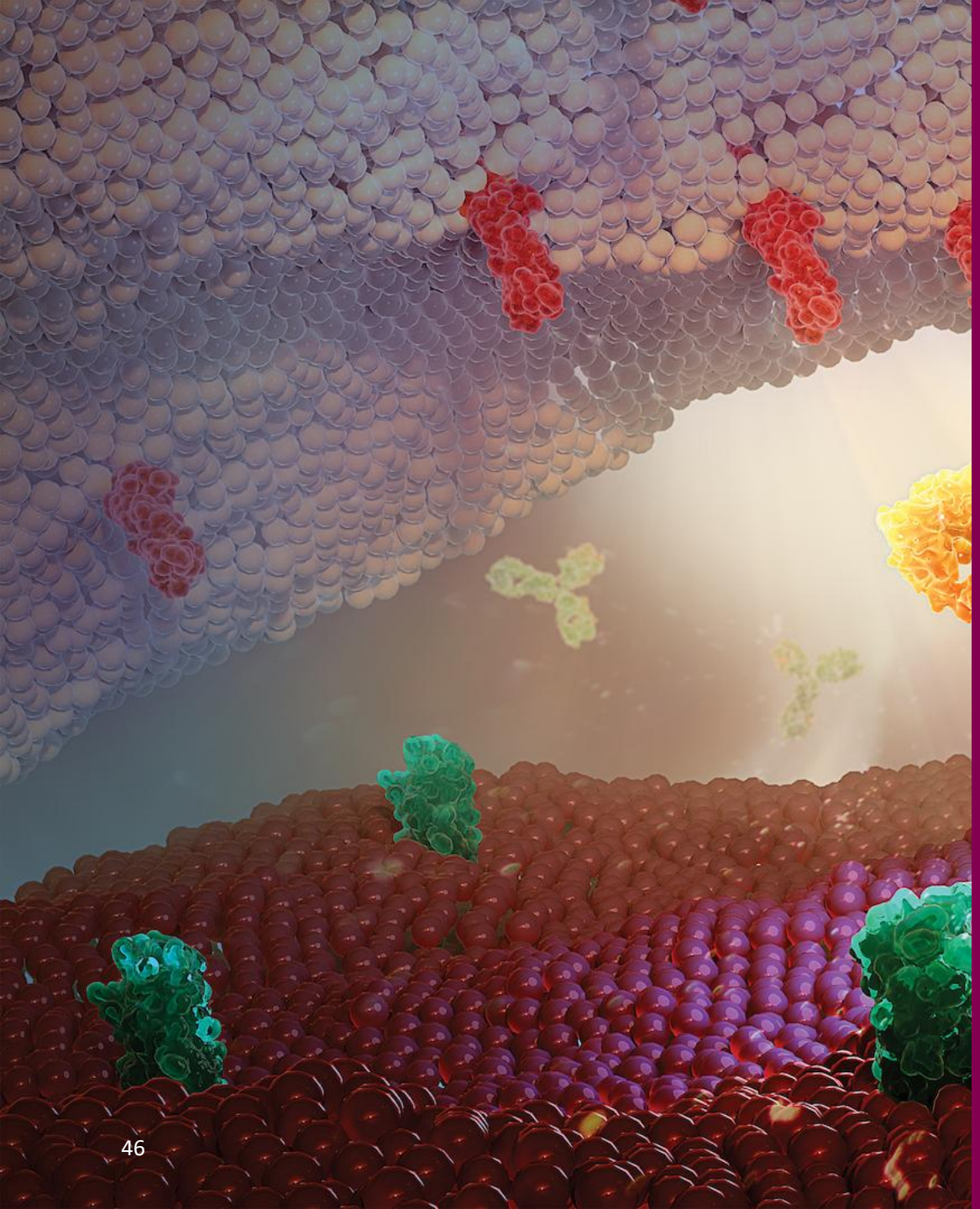
Trial	Population	Patients	Design	Endpoints	Status
Phase III eVOLVE-Cervical NCT06079671	High-risk locally advanced cervical cancer with no progression following platinum-based cCRT	800	• Randomised, double-blind, placebo-controlled, multi-centre trial • Arm 1: volrustomig • Arm 2: placebo	• Primary endpoint: PFS (Inv, ITT) • Secondary endpoints: OS, ORR, DoR	• FPCD: Q4 2023 • Data anticipated: >2026
Phase III eVOLVE-Lung02 NCT05984277	1L mNSCLC with PD-L1 <50%	900	• Double-arm randomised, open-label trial • Arm 1: volrustomig + chemotherapy • Arm 2: pembrolizumab + chemotherapy	• Primary endpoints: OS and PFS (PD-L1 < 1%) • Secondary endpoints: PFS (ITT), ORR and DoR • •	• FPCD: Q4 2023 • Data anticipated: >2026
Phase III eVOLVE-Meso NCT06097728	1L unresectable malignant pleural mesothelioma	825	• Double-arm, randomised, open-label trial • Arm 1: volrustomig + chemotherapy • Arm 2: chemotherapy or nivolumab + ipilimumab	• Primary endpoint: OS • Secondary endpoints: PFS, landmark OS, landmark PFS and ORR	• FPCD: Q4 2023 • Data anticipated: >2026
Phase III eVOLVE-HNSCC NCT06129864	Unresected, locally advanced HNSCC	1145	• Randomised, double-blind, placebo-controlled, multi-centre trial • Arm 1: volrustomig • Arm 2: observational	• Primary endpoint: PFS (BICR, PD-L1 expressing tumours) • Secondary endpoints: PFS (BICR, ITT), landmark PFS, OS (PD-L1 expressing tumours), landmark OS and OS (ITT)	• FPCD: Q1 2024 • Data anticipated: >2026

As of 29 July 2025.
Appendix: Glossary.

volrustomig (PD-1/CTLA-4 bispecific mAb)

Solid tumours

Trial	Population	Patients	Design	Endpoints	Status
Phase IIb **eVOLVE-01** **NCT06448754**	NSCLC	120	• Platform, randomised, open-label, multicenter, global trial • Arm 1A: volrustomig dose regimen 1 + chemotherapy • Arm 1B: volrustomig dose regimen 2 + chemotherapy	• Primary endpoints: safety, & tolerability, ORR • Secondary endpoints: DCR, DOR, PFS, OS	• FPCD: Q3 2024 • Data anticipated: >2026
Phase II **eVOLVE-02** **NCT06535607**	Advanced/metastatic solid tumours	50	• Platform, multi-centre trial • Sub-study 1: volrustomig monotherapy in participants with cervical cancer • Sub-study 2: volrustomig monotherapy in participants with head and neck squamous cell carcinoma	• Primary endpoints: ORR and safety • Secondary endpoints: DOR, PFS, TTR, OS, PK parameters and immunogenicity	• FPCD: Q3 2024 • Data anticipated: H2 2026
Phase II **CANTOR** **NCT06792695**	Colorectal Cancer (mCRC)	120	• Platform, randomised, open-label, multicenter, global trial • Arm A: Volrustomig + FOLFIRI + bevacizumab group • Arm B: FOLFIRI + bevacizumab group	• Primary endpoints: PFS, safety • Secondary endpoints: OS, ORR, DCR, DOR	• FPCD: Q1 2025 • Data anticipated: >2026
Phase Ib/II **eVOLVE-RCC02** **NCT07000149**	1L advanced clear cell renal cell carcinoma (ccRCC)	60	• Randomised, open-label, multicenter, global trial • Ph1b: • Arm 1 - Volrustomig Dose 1 + Casdatifan • Arm 2 - Volrustomig Dose 2 + Casdatifan	• Primary endpoints: AEs, SAEs • Secondary endpoints: ORR, DoR, PFS, DCR, PK parameters and immunogenicity, TTR	• Data anticipated H2 2026 • *Initiating*
Phase Ib **NCT04522323**	Advanced renal cell carcinoma	67	• Open-label, dose escalation and dose expansion trial • Arm 1: volrustomig + axitinib • Arm 2: volrustomig + lenvatanib	• Primary endpoints (escalation): safety, MTD, RP2D, tolerability and anti-tumour activity of combination (ORR) • Secondary endpoints: PK parameters, ADA and anti-tumour activity (PFS, OR, DoR, DCR, TTR, OS)	• FPCD: Q3 2020 • LPCD: Q2 2023 • Data anticipated: H1 2026
Phase I **NCT03530397**	Advanced solid tumours	400	• Open-label, dose escalation and dose expansion trial • Dose escalation: volrustomig i.v. • Dose expansion: volrustomig i.v. as monotherapy and + chemotherapy • Arm 1: volrustomig i.v. • Arm 2: volrustomig i.v., pemetrexed + carboplatin • Arm 3: pembrolizumab, pemetrexed + carboplatin • Arm 4: volrustomig i.v., taxane (paclitaxel or nab-paclitaxel) + carboplatin	• Primary endpoints (escalation): safety and tolerability, MTD, OBD and HPDD • Primary endpoint (expansion): antitumour activity based on ORR • Secondary endpoints: PK parameters, ADA, tumoural baseline PD-L1, anti-tumour activity (OR, DoR, DCR, PFS, OS)	• FPCD: Q2 2018 • LPCD: Q4 2023 • Data anticipated: H1 2026



Oncology:
early-stage development

AstraZeneca

FPI-2265 (PSMA radioconjugate)

Prostate cancer

Trial	Population	Patients	Design	Endpoints	Status
Phase II **AlphaBreak** **NCT06402331** **Partnered (Fusion)**	PSMA-positive mCRPC previously treated with lutetium-PSMA therapy	100	• Open-label, randomised, multi-centre trial	• Primary endpoints: PSA50 and safety	• FPCD: Q2 2024 • Data anticipated: H2 2025

As of 29 July 2025.
Appendix: Glossary.

oleclumab (CD73 mAb)

Solid tumours

Trial	Population	Patients	Design	Endpoints	Status
Phase Ib/II **NCT03611556**	Pancreatic 1L and 2L with prior gemcitabine-based chemotherapy	339	• Arm A1: gemcitabine and nab paclitaxel i.v. • Arm A2: gemcitabine and nab paclitaxel i.v. + oleclumab i.v. • Arm A3: gemcitabine and nab paclitaxel i.v. + oleclumab i.v. + *Imfinzi* i.v. • Arm B1: mFOLFOX (oxaliplatin, leucovorin, 5-FU) i.v. • Arm B2: mFOLFOX (oxaliplatin, leucovorin, 5-FU) i.v. + oleclumab i.v. • Arm B3: mFOLFOX (oxaliplatin, leucovorin, 5-FU) i.v. + oleclumab i.v. + *Imfinzi* i.v. • Global trial – US, Norway, Spain and Australia	• Primary endpoints: safety and anti-tumour activity • Secondary endpoints: PFS, PK parameters, immunogenicity, safety and anti-tumour activity	• FPCD: Q2 2018 • LPCD: Q3 2022 • Data readout: Q2 2023

As of 29 July 2025.
Appendix: Glossary.

puxitatug samrotecan (AZD8205, B7H4 ADC)

Solid tumours

Trial	Population	Patients	Design	Endpoints	Status
Phase I/II **BLUESTAR** **NCT05123482**	Breast cancer, ovarian cancer, endometrial cancer, squamous NSCLC	370	• Open-label dose escalation and expansion trial • Sub-study 1: puxitatug samrotecan monotherapy • Sub-study 2: puxitatug samrotecan + rilvegostomig	• Primary endpoints: AE, SAE, DLTs, changes in lab and preliminary efficacy parameters • Secondary endpoints: ORR, DCR, DoR, PFS, OS, PK parameters and ADA	• FPCD: Q1 2022 • Data anticipated: >2026

As of 29 July 2025.
Appendix: Glossary.

tilatamig samrotecan (AZD9592, EGFR-cMET TOP1i ADC)

Lung cancer

Trial	Population	Patients	Design	Endpoints	Status
Phase I **EGRET** **NCT05647122**	Advanced solid tumours including NSCLC, HNSCC and CRC	228	• Escalation phase, open-label, multi-centre trial • Arm 1: AZD9592 • Arm 2: AZD9592 + *Tagrisso* • Arm 3: AZD9592 + 5FU + bevacizumab	• Primary endpoints (escalation): safety and tolerability • Primary endpoints (expansion): safety, tolerability and anti-tumour activity • Secondary endpoints (escalation): PK parameters, immunogenicity and anti-tumour activity • Secondary endpoints (expansion): PK parameters and immunogenicity	• FPCD: Q1 2023 • Data anticipated: H2 2025

As of 29 July 2025.
Appendix: Glossary.

AZD0022 (KRASG12D)

Solid tumours

Trial	Population	Patients	Design	Endpoints	Status
Phase I/II **ALAFOSS-01** **NCT06599502** -	Metastatic or locally advanced colorectal cancer, pancreatic ductal adenocarcinoma and NSCLC, with KRASG12D mutation	430	• Open-label, multi-centre trial with FIH modular protocol design • Module 1: AZD0022 monotherapy with Part A (dose escalation) + Part B (dose optimisation) and Part C (potential dose expansion) • Module 2: AZD0022 + cetuximab with Part A (dose escalation) + Part B (dose optimization) and Part C (potential dose expansion)	• Primary endpoints (Part A and B): safety, tolerability and determination of MTD and/or OBD of AZD0022 as a monotherapy or combination • Primary endpoint (Part C): ORR • Secondary endpoints (Part A and B): ORR, CR rate, DoR, DCR, DRR, TTR, PFS, OS, tumour markers, ctDNA and PK parameters • Secondary endpoints (Part C): safety and tolerability, CR rate, DoR, DCR, DRR, TTR, PFS, OS, tumour markers, ctDNA and PK parameters •	• FPCD: Q4 2024 • Data anticipated: >2026

As of 29 July 2025.
Appendix: Glossary.

AZD0120 (GC012F, autologous anti-CD19 and anti-BCMA CAR-T)

Blood cancers

Trial	Population	Patients	Design	Endpoints	Status
Phase I/II NCT05850234	Relapsed/refractory multiple myeloma	68	• Open-label, single-arm, multi-centre trial	• Primary endpoints: incidence of AEs, DLTs and ORR • Secondary endpoints: DOR, PFS, OS and PK parameters	• FPCD: Q3 2023 • Data anticipated: H1 2026
Phase II	Relapsed/refractory multiple myeloma	20	• Open-label, multi-center, non-randomized trial • Dose expansion • China only	• Primary endpoint: ORR • Secondary endpoints: PFS, OS, MRD, DOR, TTR	• FPCD: • Data anticipated H1 2026

As of 29 July 2025.
Appendix: Glossary.

AZD0305 (GPRC5D ADC)

Blood cancers

Trial	Population	Patients	Design	Endpoints	Status
Phase I/IIa **NCT06106945**	R/R multiple myeloma	108	• Open-label, dose escalation and dose expansion trial • Phase I: AZD0305 prescribed at specified dose levels • Phase II: AZD0305 prescribed as RP2D	• Primary endpoints: occurrence of dose-limiting toxicities and incidence and severity of AEs and SAEs • Secondary endpoints: ORR, DoR, PFS, OS, PK parameters and immunogenicity	• FPCD: Q4 2023 • Data anticipated: H2 2026

As of 29 July 2025.
Appendix: Glossary.

AZD0754 (STEAP2 dnTGFβRII-armoured CAR-T)

Solid tumours

Trial	Population	Patients	Design	Endpoints	Status
Phase I/II **APOLLO** **NCT06267729**	Metastatic castration resistance prostate cancer with prior NHA and taxane exposure	60	• Open-label, single-arm, multi-centre trial with dose escalation and dose expansion components	• Primary endpoints (Phase I): DLT, AEs (including AESI and SAEs), determination of recommended dose for expansion phase • Secondary endpoints (Phase I): PSA related changes (PSA50, PSA90), radiological assesssment according to RECIST v1.1 and PCWG3 (ORR, BOR, DRR, DCR, TTR, rPFS, OS), PK parameters (Cmax, Tmax, Tlast, AUC)	• FPCD: Q2 2024 • Data anticipated: >2026

As of 29 July 2025.
Appendix: Glossary.

AZD1390 (ATM inhibitor)

Cancer

Trial	Population	Patients	Design	Endpoints	Status
Phase I **NCT03423628**	Recurrent glioblastoma eligible for re-irradiation, brain metastases and leptomeningeal disease, newly-diagnosed glioblastoma patients	165	• Open-label trial • Arm 1: recurrent GBM, AZD1390 + RT in dose escalation cohorts (Japan safety/PK cohorts added); optional food effect cohort initiated • Arm 3: primary GBM, AZD1390 + RT in dose escalation cohorts	• Primary endpoints: safety, tolerability and MTD • Secondary endpoints: PK parameters and preliminary assessment of anti-tumour activity	• FPCD: Q2 2018 • Data anticipated: H1 2026

As of 29 July 2025.
Appendix: Glossary.

AZD2068 (FPI-2068, EGFR cMET radioconjugate)

Solid tumours

Trial	Population	Patients	Design	Endpoints	Status
Phase I **NCT06147037**	Advanced solid tumours	110	• Multicentre, open-label dose escalation trial • Part A: optimisation of FPI-2053 dose (treatment with dose level 1 of [225Ac]-AZD2068 - fixed dose) • Part B: dose escalation of [225Ac]-AZD2068 with optimal FPI-2053	• Primary endpoints: safety and tolerability • Secondary endpoints: anti-tumour activity, immunogenicity and PK parameters	• FPCD: Q3 2024 • Data anticipated: >2026

As of 29 July 2025.
Appendix: Glossary.

AZD2284 (STEAP2 radioconjugate)

Prostate cancer

Trial	Population	Patients	Design	Endpoints	Status
Phase I **NCT06879041**	mCRPC	134	• Part A (Imaging): • Part A (Cold Antibody Exploration): aims to determine the optimal dosing regimen, with or without unconjugated antibody (AZD2275) pre-administration to improve the biodistribution of AZD2287 • Part A Expansion: aims to explore the prevalence of PSMA and STEAP2 expression by imaging • Part B (Therapeutic): • Part B (Actinium-225 Dose Escalation): aims to assess the safety, tolerability, and efficacy of escalating doses of AZD2284 informed by the optimal dosing regimen identified in Part A • Part B Expansion Cohorts 1 and 2: aims to explore efficacy of AZD2284	• Primary endpoints: safety and tolerability • Secondary endpoints: anti-tumour activity, PK parameters and immunogenicity	• FPCD: Q1 2025 • Data anticipated: >2026

As of 29 July 2025.
Appendix: Glossary.

AZD3470 (PRMT5)

Solid tumours and blood cancer

Trial	Population	Patients	Design	Endpoints	Status
Phase I **PRIMROSE** **NCT06130553**	MTAP-deficient advanced solid tumours	234	• Arm 1: AZD3470 • Global trial – 8 countries	• Primary endpoints: safety and tolerability • Secondary endpoints: PK parameters and clinical efficacy	• FPCD: Q1 2024 • Data anticipated: H2 2025
Phase I **PRIMAVERA** **NCT06137144**	R/R haematologic malignancies	110	• Modular Phase I/II open-label dose escalation and expansion trial • Module 1 – Part A (dose escalation): AZD3470 monotherapy • Module 1 – Part B (dose expansion/optimisation): AZD3470 monotherapy	• Primary endpoints: safety and tolerability • Secondary endpoints: PK parameters and clinical effacacy	• FPCD: Q1 2024 • Data anticipated: H1 2026

As of 29 July 2025.
Appendix: Glossary.

AZD4360 (CLDN18.2 ADC)

Solid tumours

Trial	Population	Patients	Design	Endpoints	Status
Phase I/II CONCLUDE NCT06921928	Histologically confirmed advanced or metastatic Pancreatic ductal adenocarcinoma (PDAC), Gastric or Gastroesophageal junction cancer (G/GEJC), and Biliary tract cancer (BTC) with documented positive CLDN18.2 expression	117	• Open-label, multi-centre trial with FIH modular protocol design • Module 1: AZD4360 monotherapy	• Primary endpoints: safety • Secondary endpoints: efficacy, PK, immunogenicity	• FPCD: Q2 2025 • Data anticipated: >2026

As of 29 July 2025.
Appendix: Glossary.

AZD5335 (anti-FRα TOP1i ADC)

Solid tumours, ovarian cancer, lung cancer

Trial	Population	Patients	Design	Endpoints	Status
Phase I/II **FONTANA** **NCT05797168**	Advanced solid tumour malignancies	446	• Module 1: AZD5335 monotherapy • Module 2: AZD5335 in combination with saruparib • Module 3: AZD5335 in combination with bevacizumab • Module 4: AZD5335 in combination with carboplatin +/- bevacizumab	• Primary endpoints: safety and tolerability • Secondary endpoints: efficacy and PK parameters	• FPCD: Q3 2023 • Data anticipated: >2026

As of 29 July 2025.
Appendix: Glossary.

AZD5492 (CD20 TITAN TCE)

Blood cancers

Trial	Population	Patients	Design	Endpoints	Status
Phase I TITANIUM NCT06542250	CLL, MCL, LBCL, FL	176	• Module 1: AZD5492 monotherapy • AZD5492 monotherapy for r/r B-cell malignancies	• Primary endpoints: safety and tolerability • Secondary endpoints: preliminary efficacy (ORR, CRR, DoR, PFS, OS), PK parameters and immunogenicity	• FPCD: Q3 2024 • Data anticipated: H2 2026

As of 29 July 2025.
Appendix: Glossary.

AZD5851 (GPC3 dnTGFβRII-armoured CAR-T)

Gastrointestinal cancer

Trial	Population	Patients	Design	Endpoints	Status
Phase I/II **ATHENA** **NCT06084884**	GPC3-positive advanced/recurrent HCC	94	• Open-label, single-arm, multi-centre trial with dose escalation and dose expansion components • AZD5851	• Primary endpoints (Phase I): DLT, AEs (including AESI and SAEs and determination of recommended dose for expansion phase • Secondary endpoints (Phase I): ORR per RECIST v. 1.1, TTR, DCR, DRR, BoR, DoR, PFS, OS and PK parameters (Cmax, Tmax, Tlast, AUC)	• FPCD: Q1 2024 • Trial disontinued due to strategic portfolio prioritsation

As of 29 July 2025.
Appendix: Glossary.

AZD5863 (CLDN18.2 CD3 bispecific antibody)

Solid tumours

Trial	Population	Patients	Design	Endpoints	Status
Phase I **NCT06005493**	Advanced or metastatic solid tumours with CLDN18.2 expression	240	• Part A: dose escalation phase to determine the safety, tolerability, RP2D, and/or MTD of AZD5863 • Part B: dose expansion phase to further characterise the safety profile and evaluate anti-tumour activity of AZD5863	• Primary endpoints (Part A): safety and tolerability • Primary endpoints (Part B): safety, tolerability and preliminary anti-tumour activity • Secondary endpoints: preliminary anti-cancer activity, PK parameters and immunogenicity	• FPCD: Q4 2023 • Data anticipated: H2 2026

As of 29 July 2025.
Appendix: Glossary.

AZD6422 (CLDN18.2 CAR-T)

Solid tumours

Trial	Population	Patients	Design	Endpoints	Status
Phase I **NCT05981235**	Advanced or metastatic CLDN18.2-positive GI tumours	96	• Open-label trial, dose escalation (Part 1) and dose expansion (Part 2)	• Primary endpoints: incidence of TEAEs, AESIs and SAEs, DLT and changes from baseline in vital signs, laboratory parameters, physical examination and 12-lead ECG • Secondary endpoints: ORR, DoR, DCR and PFS	• FPCD: Q4 2023 • Data readout: Q2 2025 • Trial discontinued due to efficacy

As of 29 July 2025.
Appendix: Glossary.

AZD7003 (GPC3 CAR-T)

Hepatocellular carcinoma (HCC)

Trial	Population	Patients	Design	Endpoints	Status
Phase I/II STARLIGHT NCT06590246	GPC3-positive advanced/recurrent HCC	121	• Open-label, single-arm, multi-centre trial with dose escalation and dose expansion components	• Primary endpoints (Phase I): DLT, AEs (including AESI and SAEs), determination of recommended dose for expansion phase • Secondary endpoints (Phase I): ORR per RECIST v. 1.1, TTR, DCR, DRR, BoR, DoR, PFS and OS; PK parameters (Cmax, Tmax, Tlast, AUC)	• FPCD: Q4 2024 • Data anticipated: >2026

As of 29 July 2025.
Appendix: Glossary.

AZD8421 (CDK2 inhibitor)

Breast cancer

Trial	Population	Patients	Design	Endpoints	Status
Phase I/II **CYCAD-1** **NCT06188520**	ER+ HER2-negative advanced breast cancer	204	• Module 1: AZD8421 • Module 2: AZD8421+ camizestrant + one or more of abemaciclib or ribociclib or palbociclib	• Primary endpoints: safety and tolerablility • Secondary endpoints: PK parameters	• FPCD: Q4 2023 • Data anticipated: H2 2026

As of 29 July 2025.
Appendix: Glossary.

AZD9574 (PARP1-sel BBB inhibitor)

Solid tumours

Trial	Population	Patients	Design	Endpoints	Status
Phase I/IIa **CERTIS-1** **NCT05417594**	Advanced solid malignancies	535	• Modular, open-label, multi-centre dose escalation and expansion trial • Module 1: AZD9574 monotherapy • Module 2: AZD9574 + temozolomide • Module 3: [11C]AZ14193391 + AZD9574 or [11C]AZ14193391 + AZD9574 + temozolomide • Module 4: AZD9574 + *Enhertu* • Module 5: AZD9574 + *Datroway*	• Primary endpoints: safety and tolerability of AZD9574 as monotherapy and in combination with anti-cancer agents, determination of PARP1 occupancy in brain by AZD9574 at examined doses and plasma concentration and evaluation of safety of radioligand [11C]AZ14193391 • Secondary endpoints: PK parameters and efficacy of AZD9574 as monotherapy and in combination with anti-cancer agents	• FPCD: Q3 2022 • Data anticipated: >2026

As of 29 July 2025.
Appendix: Glossary.

AZD9793 (GPC3 TITAN T-cell engager)

Solid tumours

Trial	Population	Patients	Design	Endpoints	Status
Phase I/II **RHEA-1** **NCT06795022**	Metastatic solid tumours	304	• Open label, non randomised, multi-centre, dose escalation and expansion • Module 1: intravenous AZD9793 • Module 2: subcutaneous AZD9793	• Primary endpoint: safety and tolerability • Secondary endpoints: ORR, BOR, DRR, DoR, TTR, PFS, OS, PK Parameters	• Data anticipated: >2026 • Initiating

As of 29 July 2025.
Appendix: Glossary.

AZD9829 (CD123 TOP1i ADC)

Blood cancers

Trial	Population	Patients	Design	Endpoints	Status
Phase I/II **ADC123** **NCT06179511**	CD123-positive haematological malignancies	104	• Open-label, multi-centre trial • Module 1: dose escalation with ascending dose level cohorts of AZD9829 in AML and MDS participants	• Primary endpoints: safety and tolerability • Secondary endpoints: PK parameters and efficacy	• FPCD: Q1 2024 • Data anticipated: >2026

As of 29 July 2025.
Appendix: Glossary.

IPH5201 (CD39 mAb)

Solid tumours

Trial	Population	Patients	Design	Endpoints	Status
Phase I **NCT04261075** **Partnered (Innate Pharma)**	Advanced solid tumours	57	• Open-label, dose escalation trial to determine MTD of IPH5201 as monotherapy, or in combination with *Imfinzi* +/- oleclumab • Part 1: IPH5201 monotherapy dose escalation to MTD • Part 2: IPH5201 + *Imfinzi* dose escalation to MTD • Part 3: IPH5201 + *Imfinzi* + oleclumab dose escalation to MTD • Route of administration: i.v. • Global trial – US and EU	• Primary endpoints: AE, SAE and DLT • Secondary endpoints: OR, DC, PK parameters and ADA	• FPCD: Q1 2020 • LPCD: Q2 2022 • Data readout: Q2 2023

As of 29 July 2025.
Appendix: Glossary.

NT-112 (KRAS G12D specific TCR)

Solid tumours

Trial	Population	Patients	Design	Endpoints	Status
Phase I **NCT06218914**	Unresectable, advanced and/or metastatic non-small cell lung cancer, colorectal adenocarcinoma, pancreatic adenocarcinoma, endometrial cancer or any solid tumour histology positive for KRAS G12D mutation	24	• Open-label, single-arm, multi-centre trial with dose escalation	• Primary endpoints: incidence of DLTs, TEAEs and SAEs • Secondary endpoints: ORR per RECIST v.1.1, BOR, DOR, CBR (CR, PR, SD), TTR, PFS and OS	• FPCD: Q1 2024 • Data anticipated: H1 2026

As of 29 July 2025.
Appendix: Glossary.

NT-125 (autologous, multi-specific neoantigen-targeting TCR-T)

Solid tumours

Trial	Population	Patients	Design	Endpoints	Status
Phase I **EudraCT: 2021-006406-73**	Adults with recurrent or metastatic NSCLC, melanoma, colorectal adenocarcinoma, HNSCC, bladder carcinoma, TNBC, cervical squamous cell carcinoma and adenocarcinoma or microsatellite instability-high/mismatch repair-deficient solid tumours	42	• Open-label, single-arm, single-centre trial with dose escalation and dose expansion components • Arm 1: NT-125	• Primary endpoint (Phase Ia): incidence of AEs defined as DLTs • Primary endpoint (Phase Ib): ORR per RECIST v.1.1 • Secondary endpoints (Phase Ia): percentage of pre-screened and enrolled subjects that receive treatment • Secondary endpoints (Phase Ib): percentage change tumour size, best percentage change tumour size, DoR, clinical benefit rate, TTP, PFS and OS	• FPCD: Q2 2023 • Trial discontinued due to strategic portfolio prioritisation

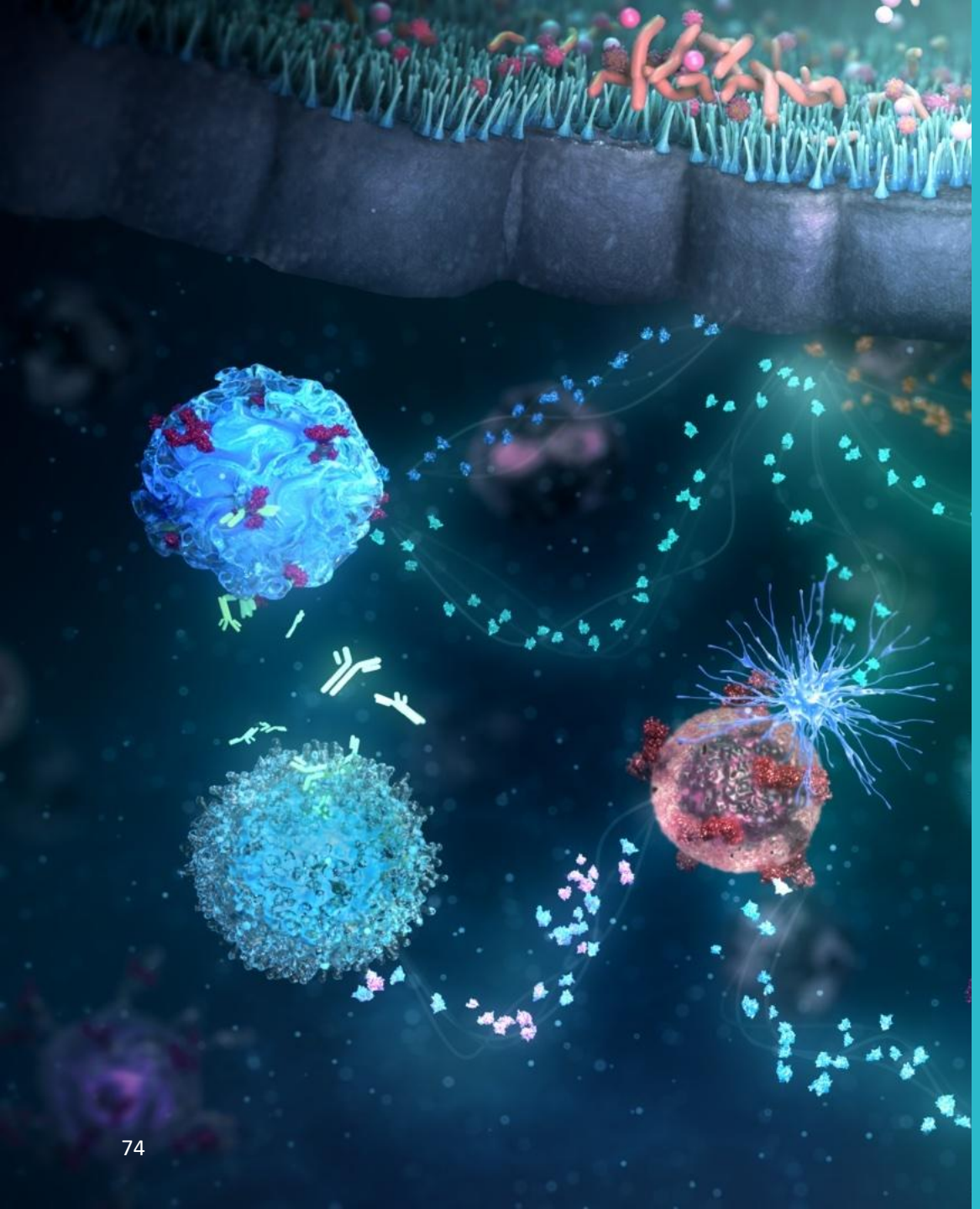
As of 29 July 2025.
Appendix: Glossary.

NT-175 (TP53-armored TCR)

Multiple cancers

Trial	Population	Patients	Design	Endpoints	Status
Phase I **NCT05877599**	Unresectable, advanced, and/or metastatic solid tumours positive for HLA-A*02:01 and TP53 R175H mutation	162	• Open-label, single-arm, multi-centre trial with dose escalation and dose expansion components	• Primary endpoint: Incidence of DLTs, TEAEs and SAEs • Secondary endpoints: ORR per RECIST v.1.1, BOR, DOR, CBR (CR, PR, SD), TTR, PFS and OS	• FPCD: Q3 2023 • Data anticipated: H2 2026

As of 29 July 2025.
Appendix: Glossary.



BioPharmaceuticals: approved medicines and late-stage development

AstraZeneca

Wainua (eplontersen, ligand-conjugated antisense)

ATTR

Trial	Population	Patients	Design	Endpoints	Status
Phase III **CARDIO-TTRansform** **NCT04136171** **Partnered (Ionis Pharmaceuticals, Inc.)**	Hereditary or wild-type transthyretin-mediated amyloid cardiomyopathy (ATTR-CM)	1438	• Arm 1: *Wainua* s.c. • Arm 2: placebo	• Primary endpoints: composite outcome of CV mortality and recurrent CV clinical events at Week 140 • Secondary endpoints: 6MWT, KCCQ, CV events and CV mortality, all cause mortality, composite outcome of CV mortality and recurrent CV clinical events in subgroup of patients treated with tafamidis at baseline	• FPCD: Q1 2020 • Data anticipated: H2 2026
Phase III **NEURO-TTRansform** **NCT04136184** **Partnered (Ionis Pharmaceuticals, Inc.)**	Hereditary transthyretin-mediated amyloid polyneuropathy (ATTRv-PN)	168	• Arm 1: *Wainua* s.c. • Arm 2: inotersen s.c.	• Primary endpoints (at Week 35): change from baseline in mNIS+7 and percent change from baseline in TTR concentration • Secondary endpoint (Week 35): changes from baseline in Norfolk QOL • Primary endpoints (at Week 66): change from baseline in mNIS+7, change from baseline in the Norfolk QoL-DN Questionnaire and percent change from baseline in TTR concentration	• FPCD: Q1 2020 • LPCD: Q3 2023 • Data readout: Q2 2022 • Co-primary endpoints met at Week 35 and Week 66
Phase III **EPIC-ATTR** **NCT06194825**	ATTR-CM	64	• Arm 1: *Wainua* s.c. Q4W • Arm 2: placebo • China only	• Primary endpoint (at week 24): percent change from baseline in serum TTR concentration • Secondary endpoints: PK, immunogenicity, disease biomarkers (NT pro-BNP, hsTnT)	• FPCD: Q4 2023 • Data anticipated: H1 2026

As of 29 July 2025.
Appendix: Glossary.

balcinrenone/dapagliflozin (MR modulator + SGLT2 inhibitor)

Heart failure, CKD

Trial	Population	Patients	Design	Endpoints	Status
Phase III **BalanceD-HF** **NCT06307652**	Heart failure patients with renal impairment (eGFR 20-60 ml/min) with heart failure event within the last 6 months	4800	• Randomised, double-blind, parallel-group, double-dummy, active-controlled, event-driven trial • Arm 1: balcinrenone/dapagliflozin 15mg/10mg • Arm 2: balcinrenone/dapagliflozin 40mg/10mg • Arm 3: dapagliflozin 10mg	• Primary endpoints: time to first occurrences of any the components of the composite of CV death, HF hospitalisation and HF event without hospitalisation • Secondary endpoints: total occurrences (first and recurrent) of the components of the composite of CV death, HF hospitalisation and HF event without hospitalisation; time to CV death; the hierarchical composite endpoint of death from any cause, total HF events, and change from baseline in KCCQ total symptom score to 24-week post-randomisation; and time do death from any cause	• FPCD: Q2 2024 • Data anticipated: >2026
Phase IIb **MIRACLE** **NCT04595370**	Heart failure with chronic kidney disease	500	• Randomised, stratified according to T2DM and eGFR (≥20 to <30 mL/min / ≥30 to <45 mL/min / ≥45 mL/min) for 12 weeks • Arm 1: AZD9977 A + dapagliflozin 10mg • Arm 2: AZD9977 B + dapagliflozin 10mg • Arm 3: AZD9977 C + dapagliflozin 10mg • Arm 4: dapagliflozin 10mg • 12 weeks	• Primary endpoint: percent change from baseline in UACR at 12 weeks • Secondary endpoints: percent change from baseline in UACR at 12 weeks to assess dose-response relationship; dose-response relationship of dapagliflozin and 3 doses of AZD9977 combined with dapagliflozin on UACR; safety, tolerability and serum potassium values; eGFR	• FPCD: Q2 2021 • LPCD: Q3 2023 • Data readout: Q4 2023
Phase IIb **MIRO-CKD** **NCT06350123**	CKD	300	• Multicentre, randomised, double-blind, dose-finding, parallel group, double-dummy trial • Arm 1: balcinrenone/dapagliflozin 15 mg/10 mg once daily • Arm 2: balcinrenone/dapagliflozin 40 mg/10 mg once daily • Arm 3: dapagliflozin 10 mg once daily	• Primary endpoint: Relative change in UACR from baseline to Week 12	• FPCD: Q2 2024 • LPCD: Q4 2024 • Data anticipated: H2 2025

As of 29 July 2025.
Appendix: Glossary.

baxdrostat (selective aldosterone synthase inhibitor)

Hypertension

Trial	Population	Patients	Design	Endpoints	Status
Phase III **BaxHTN** **NCT06034743**	Patients with uncontrolled hypertension on two or more antihypertensive medications including patients with resistant hypertension	720	• Arm 1: baxdrostat 1mg QD • Arm 2: baxdrostat 2mg QD • Arm 3: placebo QD	• Primary endpoint: effect of baxdrostat vs. placebo on seated systolic blood pressure at Week 12 • Secondary endpoints: effect of baxdrostat vs. placebo on seated systolic blood pressure at 8 weeks after randomised withdrawal, safety and tolerability	• FPCD: Q1 2024 • LPCD: Q1 2025 • Data readout: Q3 2025 • Primary endpoint met
Phase III **Bax24** **NCT06168409**	Patients with resistant hypertension on three or more antihypertensive medications	212	• Arm 1: baxdrostat 2mg QD • Arm 2: placebo QD	• Primary endpoint: effect of baxdrostat vs. placebo on ambulatory 24-hour average systolic blood pressure at Week 12	• FPCD: Q2 2024 • LPCD: Q2 2025 • Data anticipated: H2 2025
Phase III **BaxAsia** **NCT06344104**	Patients with uncontrolled hypertension on two or more antihypertensive medications including patients with resistant hypertension	300	• Arm 1: baxdrostat 1mg QD • Arm 2 baxdrostat 2mg QD • Arm 3: placebo QD	• Primary endpoint: effect of baxdrostat vs. placebo on seated systolic blood pressure at Week 12 • Secondary endpoints: effect of baxdrostat vs. placebo on ambulatory 24-hour average systolic blood pressure, safety and tolerability	• FPCD: Q2 2024 • LPCD: Q1 2025 • Data anticipated: H1 2026
Phase II **SPARK** **NCT04605549**	Patients with primary aldosteronism	18	• Arm 1: baxdrostat 2-8mg QD • US only	• Primary endpoints: safety and tolerability in patients with PA at doses from 2 to 8mg per day for 12 weeks and the reduction in SBP patients with PA after 12 weeks • Secondary endpoints: reduction in DBP as a function of dose in patients with PA after 12 weeks of treatment, change in serum potassium and requirement for potassium supplementation and change in serum sodium and requirement for fluid or mineral replacement	• FPCD: Q3 2022 • LPCD: Q2 2023 • Data readout: Q1 2025

As of 29 July 2025.
Appendix: Glossary.

baxdrostat (selective aldosterone synthase inhibitor)

Hypertension

Trial	Population	Patients	Design	Endpoints	Status
Phase II **HALO-OLE** **NCT05459688**	Patients with uncontrolled hypertension who have completed CIN-107-124	175	• Arm 1: baxdrostat 2mg QD • US only	• Primary endpoints: safety and tolerability	• FPCD: Q2 2022 • LPCD: Q3 2022 • Data readout: Q2 2024
Phase II **FigHTN** **NCT05432167**	Patients with uncontrolled hypertension and CKD	194	• Arm 1: baxdrostat (low dose) • Arm 2: baxdrostat (high dose) • Arm 3: placebo • US only	• Primary endpoint: change from baseline in mean seated systolic blood pressure vs. placebo at Week 26 • Secondary endpoint: to evaluate the treatment effect on SBP at Week 26 by dosing strategy	• FPCD: Q2 2022 • LPCD: Q2 2024 • Data readout: Q3 2024
Phase II **NCT06336356**	Patients with uncontrolled hypertension on one or more antihypertensive medications	45	• Arm 1: baxdrostat 2mg QD • Arm 2: placebo	• Primary endpoint: individual cortisol level before and after ACTH stimulation test at baseline and Week 8	• FPCD: Q2 2024 • LPCD: Q3 2024 • Data readout: Q1 2025
Phase I **NCT06194032**	Healthy volunteers	28	• Arm 1: baxdrostat 16mg (single dose) • Arm 2: baxdrostat 32mg (single dose) • Arm 3: placebo (single dose) • Arm 4: moxifloxacin 400mg (single dose)	• Primary endpoint: placebo-corrected change from baseline QTcF	• FPCD: Q1 2024 • LPCD: Q2 2024 • Data readout: Q3 2024
Phase I **NCT06357520**	Healthy volunteers	14	• Arm 1: baxdrostat 2mg and itraconazole 200mg • US only	• Primary endpoint: AUCinf and Cmax	• FPCD: Q2 2024 • LPCD: Q2 2024 • Data readout: Q3 2024
Phase I **NCT06657105**	Healthy volunteers	22	• Arm1: baxdrostat 2mg and ethiny estradiol/levonorgestrel 0.06/0.3mg	• Primary endpoints: AUCinf, AUClast and Cmax	• FPCD: Q4 2024 • LPCD: Q4 2024 • Data readout: Q2 2025

As of 29 July 2025.
Appendix: Glossary.

baxdrostat/dapagliflozin (selective ASI/SGLT2)

CKD/Prevention of heart failure

Trial	Population	Patients	Design	Endpoints	Status
Phase III **BaxDuo-Arctic** **NCT06268873**	CKD and high blood pressure	2500	• Arm 1: baxdrostat/dapagliflozin QD • Arm 2: dapagliflozin/placebo QD	• Primary endpoint: change from baseline in eGFR to post-treatment • Secondary endpoints: change from baseline in SBP and UACR, kidney HCE and eGFR	• FPCD: Q2 2024 • Data anticipated: >2026
Phase III **BaxDuo-Pacific** **NCT06742723**	CKD and high blood pressure	5000	• Arm 1: baxdrostat/dapagliflozin QD • Arm 2: dapagliflozin/placebo QD	• Primary endpoint: time to first renal composite event • Secondary endpoint: change from baseline in SBP and UACR, and CV mortality/MI/stroke/HF events	• FPCD: Q1 2025 • Data anticipated: >2026
Phase III **PREVENT-HF** **NCT06677060**	T2D, history of hypertension and established CVD and risk factor(s)	11300	• Arm 1: baxdrostat/dapagliflozin QD • Arm 2: dapagliflozin/placebo QD	• Primary endpoint: time to CV-death or first HF-events with or without hospitalisation • Secondary endpoints: time to CV death or HF-events with hospitalisation	• FPCD: Q1 2025 • Data anticipated: >2026

As of 29 July 2025.
Appendix: Glossary.

laroprovstat (AZD0780, oral PCSK9 inhibitor)

Dyslipidaemia

Trial	Population	Patients	Design	Endpoints	Status
Phase III **AZURE-LDL** **NCT07000123**	Patients with dyslipidaemia and history of clinical ASCVD or at risk for a first ASCVD event	2800	• Randomised, double-blind, placebo-controlled, parallel-group trial • Arm 1: laroprovstat • Arm 2: placebo	• Primary endpoint: • Relative change in LDL-C from baseline to 12 weeks • Secondart endpoints: • Relative change in LDL-C from baseline to 12 weeks in patients on statins • Indicator for LDL-C < 70 mg/dL (< 1.8 mmol/L) at 12 weeks	• FPCD: Q2 2025 • Data anticipated: >2026
Phase III **AZURE-HeFH** **NCT07000136**	Heterozygos familial hypercholesterolemia	405	• Randomised, double-blind, placebo-controlled, parallel-group trial • Arm 1: laroprovstat • Arm 2: placebo	• Primary endpoint: Relative change in LDL-C from baseline to 12 weeks • Secondary endpoint: Relative change in LDL-C from baseline to 12 weeks in patients on a statin, indicator for LDL-C < 70 mg/dL (< 1.8 mmol/L) at 12 weeks	• FPCD: Q2 2025 • Data anticipated: >2026
Phase III **AZURE-Outcomes** **NCT07000357**	Patients with dyslipidaemia and established ASCVD or at high risk for a first ASCVD event	15100	• Randomised, double-blind, placebo-controlled, parallel-group trial • Arm 1: laroprovstat • Arm 2: placebo	• Primary endpoint: Time to first event of any component of MACE-PLUS • Secondary endpoints: Time to first event of any component of 3P MAC, Time to first event of any component of MACE PLUS in patients with a history of ASCVD	• FPCD: Q2 2025 • Data anticipated: >2026

As of 29 July 2025.
Appendix: Glossary.

laroprovstat (AZD0780, oral PCSK9 inhibitor)

Dyslipidaemia

Trial	Population	Patients	Design	Endpoints	Status
Phase II **PURSUIT** **NCT06173570**	Dyslipidaemia	428	• Randomised trial with equal distribution across five parallel treatment arms to either placebo or one of four AZD0780 doses	• Primary endpoint: percent change in LDL-C level from baseline to Week 12 • Secondary endpoints: percent change from baseline of LDL-C at Week 12, plasma concentrations summarised by sampling timepoint, percent change from baseline at Week 12 in other lipid parameters and inflammatory markers and safety and tolerability	• FPCD: Q1 2024 • LPCD: Q2 2025 • Data readout: Q1 2025 • Primary endpoint met
Phase II **NCT06692764**	Participants with ASCVD or risk equivalents and LDL-C ≥70 mg/dL on stable medication	172	• Multi-centre, randomised, double-blind, placebo-controlled, crossover trial	• Primary endpoint: ambulatory 24-hour average systolic blood pressure at Week 4 • Secondary endpoint: ambulatory 24-hour average diastolic blood pressure at Week 4	• FPCD: Q4 2024 • Data anticipated: H2 2025
Phase II **NCT06834932**	Participants with elevated LDL-C	60	• Randomised, double-blind, placebo-controlled, multi-centre • China only	• Primary endpoint: PK parameters • Secondary endpoint: LDL-C at Week 4	• FPCD: Q4 2024 • LPCD: Q2 2025 • Data anticipated: H2 2025
Phase I **NCT06576765**	Hepatic impairment and matched healthy controls	32	• Multi-centre, single-dose, non-randomised, open-label, parallel-group trial	• Primary endpoint: PK parameters • Secondary endpoints: safety and tolerability	• FPCD: Q3 2024 • LPCD: Q4 2024 • Data readout: Q2 2025
Phase I **NCT06592482**	Renal impairment and matched healthy controls	30	• Multi-centre, single-dose, non-randomised, open-label, parallel-group trial	• Primary endpoint: PK parameters • Secondary endpoints: safety and tolerability	• FPCD: Q3 2024 • LPCD: Q4 2024 • Data readout: Q2 2025
Phase I **NCT06671405**	Healthy volunteers	78	• Open-label, fixed sequence trial to assess the PK of AZD0780 when administered in combination with itraconazole, carbamazepine, and the PK of midazolam and EE/LNG when administered with AZD0780	• Primary endpoint: PK parameters • Secondary endpoints: safety and PK parameters	• FPCD: Q4 2024 • LPCD: Q1 2025 • Data anticipated: H2 2025
Phase I **NCT06742853**	Healthy volunteers with elevated LDL-C	120	• Randomised, single-blind, placebo-controlled trial	• Primary endpoints: percent change in LDL-C at Week-4 and safety and tolerability	• FPCD: Q4 2024 • Data anticipated: H2 2025

As of 29 July 2025.
Appendix: Glossary.

zibotentan/dapagliflozin (ETA receptor antagonist/SGLT2 inhibitor)

Chronic kidney disease

Trial	Population	Patients	Design	Endpoints	Status
Phase III **ZENITH High Proteinuria** **NCT06087835**	CKD and high proteinuria	1835	• Randomised, parallel, multi-centre, double-blind trial • Arm 1: zibotentan/dapagliflozin dose A or dose B • Arm 2: dapagliflozin	• Primary endpoint: change in eGFR from baseline • Secondary endpoints: change in UPCR from baseline to each participant's mean level; change in UACR from baseline to each participant's mean level; time to the first occurrence of any of the components of the renal composite endpoint of 40% sustained decline in eGFR or ESKD or renal death	• FPCD: Q4 2023 • LPCD: Q4 2024 • Data anticipated: >2026

As of 29 July 2025.
Appendix: Glossary.

zibotentan/dapagliflozin (ETA receptor antagonist/SGLT2 inhibitor)

Liver cirrhosis

Trial	Population	Patients	Design	Endpoints	Status
Phase II **ZEAL** **NCT05516498**	Part A: participants with Child-Pugh A cirrhosis with features of portal hypertension and with no history of decompensation events Part B: participants with a broader range of Child-Pugh A and Child-Pugh B cirrhosis with more severe disease	195	• Phase IIa/b multi-centre, randomised, double-blind, placebo-controlled, parallel group dose-ranging trial • Part A - Arm 1: placebo • Part A - Arm 2: zibotentan dose B + dapagliflozin • Part B - Arm 1: placebo • Part B - Arm 2: placebo + dapagliflozin • Part B - Arm 3: zibotentan dose A + dapagliflozin • Part B - Arm 4: zibotentan dose B + dapagliflozin • Part B - Arm 5: zibotentan dose C + dapagliflozin	• Primary endpoint (Part A): absolute change in HVPG from baseline to Week 6 comparing zibotentan and dapagliflozin in combination vs. placebo • Primary endpoint (Part B): absolute change in HVPG from baseline to Week 6 comparing zibotentan and dapagliflozin in combination and dapagliflozin mono vs. placebo	• FPCD: Q4 2022 • Data readout: Q2 2025 • Trial discontinued due to efficacy

As of 29 July 2025.
Appendix: Glossary.

Airsupra (PT027, SABA/ICS, pMDI)

Asthma

Trial	Population	Patients	Design	Endpoints	Status
Phase IIIb **BATURA** **NCT05505734** **Managed by Avillion (Avillion)**	Adults and adolescents with mild asthma	2517	• Randomised, double-blind, multi-centre, parallel-group, decentralised • 12 to 52-week treatment period • Arm 1: *Airsupra* MDI 160/180µg • Arm 2: AS MDI 180µg • US only	• Primary endpoint: time to first severe asthma exacerbation	• FPCD: Q3 2022 • LPCD: Q1 2024 • Data readout: Q4 2024 • Primary endpoint met
Phase IIIb **ACADIA** **NCT06307665** -	Adolescents with asthma	440	• Randomised, double-blind, multi-center, parallel-group • Arm 1: BDA MDI 160/180µg prn • Arm 2: AS MDI 180µg prn	• Primary endpoint: severe asthma exacerbation rate (annualised) • Secondary endpoints: time to first severe exacerbation, annualised total systemic corticosteroid exposure, safety (AEs and SAEs), PK sub-study (including Cmax, AUClast and AUCinf)	• FPCD: Q2 2024 • Data anticipated: >2026
Phase III **BAIYUN** **NCT06471257**	Adult patients with asthma	790	• Randomised, double-blind, multi-centre, event-driven, parallel-group • Arm 1: BDA MDI 160/180µg prn • Arm 2: AS MDI 180µg prn • China only	• Primary endpoint: time to first severe exacerbation • Secondary endpoints: severe exacerbation rate (annualised), total systemic corticosteroid exposure, ACQ-5 responder, AQLQ+12 responder	• FPCD: Q3 2024 • Data anticipated: H2 2026
Phase II **MITCHELL** **NCT06644924** -	Adults with asthma	102	• Randomised, single-dose, double-blind, placebo-controlled, 3-period, 3-treatment, crossover, multicenter • Arm 1: AS MDI 180µg (double-blind) • Arm 2: Placebo MDI (double-blind) • Arm 3: Ventolin Evohaler 200µg (open-label) • US Only	• Primary endpoint: Mean change from baseline in FEV1 AUC0-6 (Non-inferiority of AS MDI relative to Ventolin Evohaler) • Secondary endpoints: FEV1 AUC0-6, Mean change from baseline in FEV1 AUC0-4, Safety (AEs and SAEs)	• FPCD: Q1 2025 • LPCD: Q2 2025 • Data anticipated: H2 2025
Phase I **PUTUO** **NCT06514157**	Healthy volunteers	14	• Open-label, single-dose, single-centre trial • Treatment: BDA MDI 160µg/180µg (single dose)	• Primary endpoints: PK parameters for budesonide and albuterol include AUClast, AUCinf, Cmax, tmax, tlast, t½λz, CL/F and Vz/F	• FPCD: Q3 2024 • LPCD: Q3 2024 • Data readout: Q1 2025

As of 29 July 2025.
Appendix: Glossary.

Breztri, Trixeo (LAMA/LABA/ICS)

Asthma

Trial	Population	Patients	Design	Endpoints	Status
Phase III **KALOS** **NCT04609878**	Uncontrolled asthma	2266	• Randomised, double-blind, double-dummy, parallel group and multi-centre trial • Treatments (24- to 52-week variable length) • Arm 1: BGF 320/28.8/9.6µg BID MDI • Arm 2: BGF 320/14.4/9.6µg BID MDI • Arm 3: *Symbicort* Aerosphere 320/9.6µg BID MDI • Arm 4: *Symbicort* 320/9µg BID pMDI	• Primary endpoint: change from baseline in FEV1 AUC0-3 at Week 24 • Secondary endpoint: change from baseline in morning pre-dose trough FEV1 at Week 24	• FPCD: Q1 2021 • Data readout: Q2 2025
Phase III **LOGOS** **NCT04609904**	Uncontrolled asthma	2182	• Randomised, double-blind, double dummy, parallel group and multi-centre trial • Treatments (24- to 52-week variable length) • Arm 1: BGF 320/28.8/9.6µg BID MDI • Arm 2: BGF 320/14.4/9.6µg BID MDI • Arm 3: *Symbicort* Aerosphere 320/9.6µg BID MDI • Arm 4: *Symbicort* 320/9µg BID pMDI	• Primary endpoint: change from baseline in FEV1 AUC0-3 at Week 24 • Secondary endpoint: change from baseline in morning pre-dose trough FEV1 at Week 24	• FPCD: Q1 2021 • Data readout: Q2 2025
Phase III **VATHOS** **NCT05202262**	Inadequately controlled asthma despite treatment with medium dose ICS or ICS/LABA	645	• Randomised, double-blind, parallel group, multi-centre trial • Treatments (24-week) • Arm 1: *Symbicort* Aerosphere 320/9.6µg BID MDI • Arm 2: PT009 160/9.6µg BID MDI • Arm 3: BD 320µg BID MDI • Arm 4:open-label *Symbicort* Turbuhaler 320/9µg BID •	• Primary endpoint: change from baseline in FEV1 AUC0-3 at Week 24	• FPCD: Q1 2022 • Data readout: Q2 2025
Phase III **LITHOS** **NCT05755906**	Inadequately controlled asthma despite treatment with low dose ICS or ICS/LABA	373	• Randomised, double-blind, parallel group and multi-centre • Treatments (12-week) • Arm 1: PT009 160/9.6µg BID MDI • Arm 2: BD 160µg BID MDI	• Primary endpoint: Change from baseline in forced expiratory volume in 1 second (FEV1) area under the curve 0 to 3 hours (AUC0-3) at Week 12	• FPCD: Q1 2023 • Data readout: Q1 2025

As of 29 July 2025.
Appendix: Glossary.

Breztri, Trixeo (LAMA/LABA/ICS)

COPD

Trial	Population	Patients	Design	Endpoints	Status
Phase III **ATHLOS** **NCT06067828**	COPD	180	• Randomised, double-blind, three-treatment, three-period, crossover trial • Treatments (2-week treatment periods, 2-week washout between treatments) • Arm 1: *Breztri* 320/14.4/9.6µg BID MDI • Arm 2: *Symbicort* Aerosphere 320/9.6µg BID MDI • Arm 3: placebo BID MDI	• Primary endpoint: change from baseline in isotime IC • Secondary endpoint: change from baseline in constant work rate cycle ergometry endurance time	• FPCD: Q4 2023 • Data anticipated: H1 2026
Phase III **THARROS** **NCT06283966**	COPD	5000	• Randomised, double blind, parallel group, multi-centre event-driven trial comparing BGF MDI 320/14.4/9.6µg BID with GFF MDI 14.4/9.6µg BID in participants with COPD who are at risk of a cardiopulmonary event	• Primary endpoint: time to first severe cardiac or COPD event • Secondary endpoints: time to first severe COPD exacerbation event, time to first severe cardiac event, time to cardiopulmonary death, moderate/severe COPD exacerbation rate, time to MI hospitalisation or cardiac death and time to HF acute healthcare visit/hospitalisation or cardiac death	• FPCD: Q1 2024 • Data anticipated: >2026

As of 29 July 2025.
Appendix: Glossary.

Fasenra (IL-5R mAb)

Nasal polyposis and other eosinophilic diseases

Trial	Population	Patients	Design	Endpoints	Status
Phase III **MANDARA** **NCT04157348**	Patients with r/r EGPA on corticosteroid therapy with or without stable immunosuppressive therapy; age 18 years and older	140	• Arm 1: *Fasenra* 30mg Q4W s.c. • Arm 2: mepolizumab 300mg Q4W s.c. • 52-week trial with a minimum 1-year open label extension	• Primary endpoint: proportion of patients achieving remission (BVAS=0 and OCS dose ≤4mg/day) at Week 36 and Week 48	• FPCD: Q4 2019 • LPCD: Q3 2022 • Data readout: Q3 2023 • Primary endpoint met
Phase III **NATRON** **NCT04191304**	Patients with HES (history of persistent eosinophilia >1500 cells/µL with evidence of end organ manifestations attributable to eosinophilia) and signs or symptoms of HES worsening/flare at Visit 1; age 12 years and older	134	• Arm 1: *Fasenra* 30mg Q4W s.c. • Arm 2: placebo Q4W s.c. • 24-week trial with a minimum 1-year open label extension	• Primary endpoint: time to first HES worsening/flare	• FPCD: Q3 2020 • LPCD: Q4 2024 • Data readout: Q2 2025 • Primary endpoint met

As of 29 July 2025.
Appendix: Glossary.

Fasenra (IL-5R mAb)

Severe, uncontrolled asthma and COPD

Trial	Population	Patients	Design	Endpoints	Status
Phase III **RESOLUTE** **NCT04053634**	Patients with moderate to very severe COPD with a history of frequent exacerbations on a background triple therapy (ICS/LABA/LAMA); age 40 to 85 years	689	• Double-blind, placebo-controlled trial • Arm 1: *Fasenra* 100mg Q8W s.c. • Arm 2: placebo Q8W s.c. • 56-week treatment	• Primary endpoint: annualised rate of moderate or severe exacerbations over 56 weeks	• FPCD: Q4 2019 • LPCD: Q3 2024 • Data anticipated: H2 2025

As of 29 July 2025.
Appendix: Glossary.

Saphnelo (type I interferon receptor mAb)

Lupus (SLE/LN)

Trial	Population	Patients	Design	Endpoints	Status
Phase III **TULIP-SC** **NCT04877691** **Partnered (BMS)**	Moderate to severe SLE	360	• Arm 1: *Saphnelo* s.c. • Arm 2: placebo s.c.	• Primary endpoint: BICLA at Week 52	• FPCD: Q3 2021 • LPCD: Q3 2024 • Data anticipated: H2 2025
Phase III **AZALEA-SLE** **NCT04931563** **Partnered (BMS)**	Moderate to severe SLE	276	• Arm 1: 300mg *Saphnelo* i.v. Q4W • Arm 2: placebo i.v. Q4W • Asia only	• Primary endpoint: BICLA at Week 52	• FPCD: Q4 2021 • LPCD: Q2 2024 • Data readout: Q2 2025 • Primary endpoint met
Phase III **IRIS** **NCT05138133** **Partnered (BMS)**	Active, proliferative LN	360	• Arm 1: *Saphnelo* i.v. • Arm 2: placebo i.v.	• Primary endpoint: CRR at Week 52	• FPCD: Q2 2022 • Data anticipated: H2 2026
Phase III **LAVENDER** **NCT06015737** **Partnered (BMS)**	Chronic and/or subacute CLE	460	• Arm 1: *Saphnelo* s.c. • Arm 2: placebo s.c.	• Primary endpoint (US): CLA-IGA-R erythema 0/1 at Week 24 • Primary endpoint (EU and RoW): CLASI-70 at Week 24	• FPCD: Q4 2024 • Data anticipated: >2026

As of 29 July 2025.
Appendix: Glossary.

Saphnelo (type I interferon receptor mAb)

Sclerosis and other myopathies

Trial	Population	Patients	Design	Endpoints	Status
Phase III **DAISY** **NCT05925803** **Partnered (BMS)**	Systemic sclerosis	306	• Arm 1: *Saphnelo* s.c. • Arm 2: placebo s.c.	• Primary endpoint: CRISS-25 at Week 52	• FPCD: Q4 2023 • Data anticipated: H2 2026
Phase III **JASMINE** **NCT06455449** **Partnered (BMS)**	Idiopathic inflammatory myopathies	240	• Arm 1: *Saphnelo* s.c. • Arm 2: placebo s.c.	• Primary endpoint: Total Improvement Score ≥40 at Week 52	• FPCD: Q4 2024 • Data anticipated: >2026

As of 29 July 2025.
Appendix: Glossary.

Tezspire (TSLP mAb)

CRSwNP, COPD and EoE

Trial	Population	Patients	Design	Endpoints	Status
Phase III **WAYPOINT** **NCT04851964** **Partnered (AMGEN)**	Severe chronic rhinosinusitis with nasal polyps; age 18 years and older	416	• Arm 1: *Tezspire* s.c. • Arm 2: placebo s.c. • 52-week trial	• Co-primary endpoint: nasal polyp score and participant reported nasal congestion	• FPCD: Q2 2021 • LPCD: Q4 2023 • Data readout: Q4 2024 • Co-primary endpoints met
Phase III **CROSSING** **NCT05583227** **Partnered (AMGEN)**	Adult and paediatric aged 12 years and older with eosinophilic esophagitis	360	• Arm 1: *Tezspire* s.c. low dose • Arm 2: *Tezspire* s.c. high dose • Arm 3: placebo • 52-week trial	• Co-primary endpoints: histologic response of peak esophageal eosinophil per HPF count of ≤6 across all available esophageal levels and change from baseline in Dysphagia Symptom Questionnaire score	• FPCD: Q1 2023 • LPCD: Q3 2025 • Data anticipated: H2 2026
Phase III **EMBARK** **NCT06883305** **Partnered (Amgen)**	Adults with moderate to very severe COPD	990	• Randomized, double-blind, placebo-controlled • Arm 1: *Tezspire* s.c. dose 1 • Arm 2: *Tezspire* s.c. dose 2 • Arm 3: placebo • 52-week treatment minimum	• Primary endpoint: Annualized moderate to severe COPD exacerbations.	• FPCD: Q2 2025 • Data anticipated: >2026
Phase III **JOURNEY** **NCT06878261** **Partnered (Amgen)**	Adults with moderate to very severe COPD	990	• Randomized, double-blind, placebo-controlled • Arm 1: *Tezspire* s.c. dose 1 • Arm 2: *Tezspire* s.c. dose 2 • Arm 3: placebo • 52-week treatment minimum	• Primary endpoint: Annualized moderate to severe COPD exacerbations	• FPCD: Q2 2025 • Data anticipated: >2026

As of 29 July 2025.
Appendix: Glossary.

Tezspire (TSLP mAb)

Severe, uncontrolled asthma

Trial	Population	Patients	Design	Endpoints	Status
Phase III **NAVIGATOR** **NCT03347279** **Partnered (AMGEN)**	Severe asthma; age 12 to 80 years	1061	• Arm 1: *Tezspire* s.c. • Arm 2: placebo s.c. • 52-week trial	• Primary endpoint: annual asthma exacerbation rate • Secondary endpoints: change from baseline in pre-BD FEV1, asthma related QoL (AQLQ(S)+12) and asthma control (ACQ-6)	• FPCD: Q1 2018 • LPCD: Q3 2019 • Data readout: Q4 2020 • Primary endpoint met
Phase III **DIRECTION** **NCT03927157** **Partnered (AMGEN)**	Severe asthma; age 18 to 80 years	405	• Arm 1: *Tezspire* s.c. • Arm 2: placebo s.c. • 52-week trial • Regional trial (Asia) – 3 countries	• Primary endpoint: annual asthma exacerbation rate • Secondary endpoints: change from baseline in pre-BD FEV1, asthma related QoL (AQLQ(S)+12) and asthma control (ACQ-6)	• FPCD: Q3 2019 • LPCD: Q2 2023 • Data readout: Q3 2024 • Primary endpoint met

As of 29 July 2025.
Appendix: Glossary.

tozorakimab (IL-33 ligand mAb)

COPD

Trial	Population	Patients	Design	Endpoints	Status
Phase III **OBERON** **NCT05166889**	Adults with symptomatic COPD with a history of exacerbations	1132	• Randomised, double-blind, placebo-controlled, parallel-group • Treatment: 52-week • Arm 1: tozorakimab dose 1 s.c. + SoC • Arm 2: tozorakimab dose 2 s.c. + SoC • Arm 3: placebo s.c. + SoC •	• Primary endpoint: annualised rate of moderate to severe COPD exacerbations (former smokers) • Secondary endpoints: annualised rate of moderate to severe COPD exacerbations (former or current smokers) and change in pre-BD FEV1, E-RS:COPD and SGRQ	• FPCD: Q1 2022 • Data anticipated: H1 2026
Phase III **TITANIA** **NCT05158387**	Adults with symptomatic COPD with a history of exacerbations	1174	• Randomised, double-blind, placebo-controlled, parallel-group • Treatment: 52-week • Arm 1: tozorakimab dose 1 s.c. + SoC • Arm 2: tozorakimab dose 2 s.c. + SoC • Arm 3: placebo s.c. + SoC	• Primary endpoint: annualised rate of moderate to severe COPD exacerbations (former smokers) • Secondary endpoints: annualised rate of moderate to severe COPD exacerbations (former or current smokers) and change in pre-BD FEV1, E-RS:COPD and SGRQ	• FPCD: Q1 2022 • Data anticipated: H1 2026
Phase III **PROSPERO** **NCT05742802**	Subjects who completed either OBERON or TITANIA will be offered the opportunity to consent (adults with symptomatic COPD with a history of exacerbations)	1596	• Randomised, double-blind, placebo-controlled, parallel-group, long-term extension trial • Treatment: 52-weeks • Arm 1: tozorakimab dose 1 s.c. + SoC • Arm 2: tozorakimab dose 2 s.c. + SoC • Arm 3: placebo s.c. + SoC	• Primary endpoint: annualised rate of severe COPD exacerbation in primary population of former smokers over the treatment period incorporating both the predecessor studies and PROSPERO • Secondary endpoint: annualised rate of severe COPD exacerbation in the overall population of current and former smokers	• FPCD: Q1 2023 • Data anticipated: H2 2026
Phase III **MIRANDA** **NCT06040086**	Adults with symptomatic COPD with a history of exacerbations	1454	• Randomised, double-blind, placebo-controlled, parallel group • Arm 1: tozorakimab dose s.c. + SoC • Arm 2: placebo s.c. + SoC	• Primary endpoint: annualised rate of moderate to severe COPD exacerbations (former smokers) • Secondary endpoints: annualised rate of moderate to severe COPD exacerbations (former or current smokers), annualised rate of severe COPD exacerbations (former/former or current smokers) and change in pre-BD FEV1, E-RS:COPD and SGRQ	• FPCD: Q4 2023 • Data anticipated: H1 2026

As of 29 July 2025.
Appendix: Glossary.

tozorakimab (IL-33 ligand mAb)

Severe viral LRTD, asthma

Trial	Population	Patients	Design	Endpoints	Status
Phase III **TILIA** **NCT05624450**	Adults hospitalised for viral lung infection requiring supplemental oxygen	2870	• Randomised, double-blind, placebo-controlled, parallel group • Arm 1: tozorakimab dose i.v. + SoC • Arm 2: placebo i.v. + SoC	• Primary endpoint: progression to death or to invasive mechanical ventilation/extracorporeal membrane oxygenation • Secondary endpoints: safety and other efficacy measures	• FPCD: Q4 2022 • Data anticipated: H2 2026
Phase II **UMBRIEL** **NCT06932263**	Adult participants with uncontrolled asthma on medium-to-high dose inhaled corticosteroids	540	• Multi-centre, double-blind, placebo-controlled dose range finding • Arm 1: tozorakimab dose 1 s.c. • Arm 2: tozorakimab dose 2 s.c. • Arm 3: placebo s.c.	• Primary endpoint: annualised rate of severe asthma exacerbations • Secondary endpoints: annualised rate of severe asthma exacerbations, time-to-first severe asthma exacerbation; pre and post BD FEV1, change in baseline ACQ- 6 and AQLQ(S), safety and other efficacy measures	• FPCD: Q2 2025 • Data anticipated: >2026 • Enrolling

As of 29 July 2025.
Appendix: Glossary.

Next-generation propellant (NGP)

pMDI

Trial	Population	Patients	Design	Endpoints	Status
Phase III **NCT05755932**	Mucociliary clearance in healthy volunteers	30	• Randomised, double-blind, multi-site, two-way crossover trial with propellant only • Arm 1: NGP pMDI; 6 inhalations BID for 7 days • Arm 2: HFA pMDI; 6 inhalations BID for 7 days	• Primary endpoint: change from baseline in MCC through 60 minutes following inhalation of 99m technetium sulfur colloid and gamma camera imaging • Secondary endpoint: change from baseline in MCC at 3 hours following inhalation of 99m technetium sulfur colloid and gamma camera imaging	• FPCD: Q2 2023 • Data readout: Q4 2024
Phase III **NCT05850494**	Well-controlled or partially-controlled asthma	52	• Randomised, mulit-centre double-blind, single-dose crossover trial • Arm 1: NGP propellant only pMDI; 4 inhalations per dose • Arm 2: HFA propellant only pMDI; 4 inhalations per dose	• Primary endpoints: change from baseline FEV1 0 to 15 minutes post-dose, cumulative incidence of bronchospasm events and safety and tolerability	• FPCD: Q2 2023 • Data readout: Q1 2024 • Primary endpoint met
Phase III **NCT06075095**	COPD	300	• Randomised, placebo-controlled, double-blind, multi-centre, 4-week, 3-way crossover pharmacodynamic trial to assess the equivalence of *Breztri* delivered by pMDI NGP vs. with *Breztri* delivered by MDI HFA • Arm 1: *Breztri* pMDI NGP 320/14.4/9.6µg • Arm 2: *Breztri* pMDI HFA 320/14.4/9.6µg • Placebo: MDI HFA	• Primary endpoints: changes in FEV1 AUC (0-4) and change in morning pre-dose trough FEV1 • Secondary endpoints: safety and efficacy	• FPCD: Q1 2024 • Data anticipated: H2 2025
Phase III **NCT06502366**	Asthma	398	• Randomised, placebo-controlled, double-blind, multi-centre, 12-week, 3-way, partial-replicate crossover trial • BDA MDI NGP 160/180µg • BDA MDI HFA 160/180µg • Placebo: MDI HFA	• Primary endpoint: change from baseline in peak FEV1 in 0-60 minutes after dosing at Day 29 • Secondary endpoint: change from baseline in morning pre-dose trough FEV1	• FPCD: Q3 2024 • Data anticipated: H1 2026
Phase III **NCT05573464**	Moderate to very severe COPD	542	• Randomised, double-blind, 12-week (with an extension to 52 weeks in a subset of participants), parallel-group, multi-centre trial • Arm 1: *Breztri* MDI NGP 160/7.2/4.8µg (2 inhalations BID) • Arm 2: *Breztri* MDI HFA 160/7.2/4.8µg (2 inhalations BID)	• Primary endpoints: number of participants with AEs/SAEs and potentially clinically significant changes in Digital 12-lead Holter ECG, laboratory values, blood pressure, pulse rate, respiratory rate and body temperature	• FPCD: Q3 2022 • Data readout: Q4 2024

As of 29 July 2025.
Appendix: Glossary.

Next-generation propellant (NGP)

pMDI

Trial	Population	Patients	Design	Endpoints	Status
Phase I **NCT05477108**	Healthy volunteers	108	• Randomised, double-blind, single-dose, single-centre, partial-replicate, 3-way crossover trial • Arm 1: *Breztri* pMDI NGP 160/7.2/4.8µg (single dose of 4 inhalations) • Arm 2: *Breztri* pMDI HFA 160/7.2/4.8µg (single dose of 4 inhalations)	• Primary endpoints: AUCinf, AUClast and Cmax	• FPCD: Q3 2022 • LPCD: Q1 2023 • Data readout: Q4 2023 • Primary endpoint met
Phase I **NCT05569421**	Healthy volunteers	108	• Randomised, double-blind, single-dose, single-centre, partial-replicate, 3-way crossover trial • Arm 1: *Breztri* pMDI NGP 160/7.2/4.8µg (single dose of 4 inhalations) • Arm 2: *Breztri* pMDI HFA 160/7.2/4.8µg (single dose of 4 inhalations)	• Primary endpoints: AUCinf, AUClast and Cmax	• FPCD: Q4 2022 • LPCD: Q2 2023 • Data readout: Q1 2024 • Primary endpoint met
Phase I **NCT06139991**	Healthy volunteers	66	• Randomised, double-blind, single-dose, crossover trial to assess the equivalence of *Airsupra* delivered by pMDI NGP vs. with *Airsupra* delivered by pMDI HFA • Arm 1: *Airsupra* pMDI NGP 80/90µg (single dose of 2 inhalations) • Arm B: *Airsupra* pMDI HFA 80/90µg (single dose of 2 inhalations)	• Primary endpoints: AUClast and Cmax	• FPCD: Q4 2023 • LPCD: Q2 2024 • Data readout: Q4 2024
Phase I **NCT06297668**	Healthy volunteers	42	• Randomised, partial double-blind, single dose, three-way crossover trial • Arm 1: BGF MDI HFA 160/7.2/4.8µg with spacer • Arm 2: BGF MDI NGP 160/7.2/4.8µg with spacer • Arm 3: BGF MDI NGP 160/7.2/4.8µg without spacer	• Primary endpoints: AUClast of BGF MDI and Cmax of BGF MDI	• FPCD: Q2 2024 • LPCD: Q2 2024 • Data readout: Q4 2024 • Primary endpoint met
Phase I **NCT06723756**	Healthy volunteers	105	• Randomised, double-blind, single-dose, single-centre, 3-way crossover trial • Arm 1: *Breztri* pMDI NGP 160/14.4/4.8µg (single dose of 2 inhalations) • Arm 2: *Breztri* pMDI HFA 160/14.4/4.8µg (single dose of 2 inhalations)	• Primary endpoints: AUClast and Cmax	• FPCD: Q1 2025 • Data anticipated: H2 2025

As of 29 July 2025.
Appendix: Glossary.

Beyfortus (nirsevimab, RSV mAb-YTE)

Infection

Trial	Population	Patients	Design	Endpoints	Status
Phase III **CHIMES** **NCT05110261**	Healthy infants (born 29 weeks 0 days or greater gestational age)	800	• Randomised, double-blind, placebo-controlled • Arm 1: *Beyfortus* i.m. • Arm 2: placebo i.m. • China only	• Primary endpoint: efficacy • Secondary endpoints: safety, PK parameters and ADA	• FPCD: Q4 2021 • LPCD: Q4 2024 • Data anticipated: H1 2026

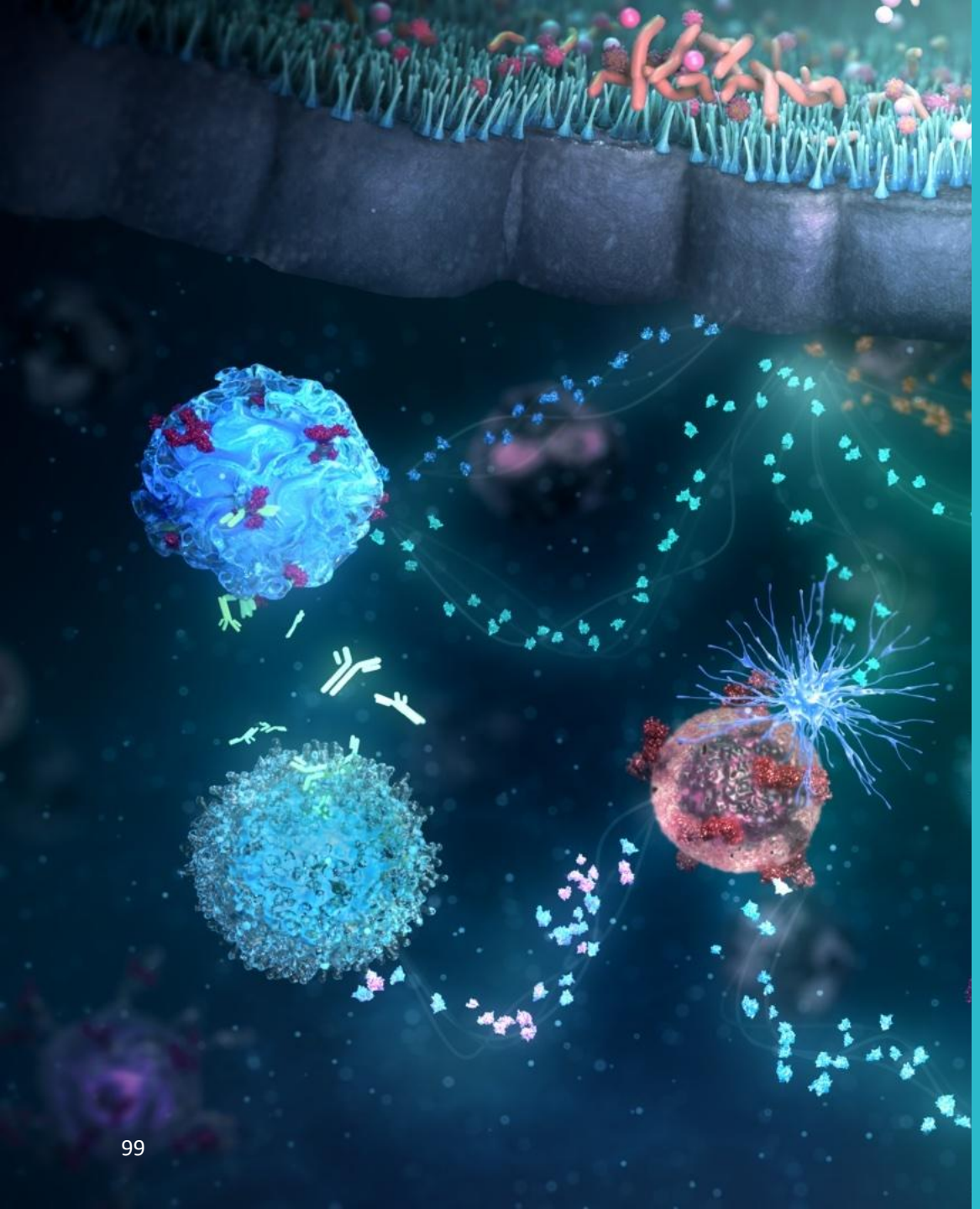
As of 29 July 2025.
Appendix: Glossary.

Kavigale (sipavibart, SARS-CoV-2 LAAB)

COVID-19

Trial	Population	Patients	Design	Endpoints	Status
Phase III **SUPERNOVA** **NCT05648110**	Phase I: healthy adults; age 18 to 55 years Phase II: immuocompetent or immunoimpared adults Phase III: 12 years of age or older with conditions causing immune impairment	3200	• 2 parts (Phase I: sentinel safety cohort and Phase III: main cohort) • Phase I (sentinel safety cohort): 56 healthy adults, age 18 to 55 years, randomised in a 5:2 ratio to receive AZD5156 or placebo • Phase III (main cohort): randomised 1:1 to receive AZD3152 300mg or comparator (600mg *Evusheld* or placebo) administered i.m. in the anterolateral thigh on Day 1; participants will receive a second dose of their original randomised trial intervention 6 months after Visit 1 • Phase II (sub-study, open-label): participants randomised 2:1 to receive 1200mg i.v. AZD3152 or 300mg i.m. *Evusheld*	• Primary endpoints (Phase III main cohort): to evaluate the safety of AZD3152 and Evusheld and/or placebo and to compare the efficacy of AZD3152 to Evusheld and/or placebo in the prevention of symptomatic COVID-19 • Primary endpoints (Phase II sub-study): to evaluate the safety of AZD3152 and Evushled; to compare the nAb responses to the SARS-CoV-2 to a current variant of concern following AZD3152 administration vs. SARS-CoV-2 nAb responses to prior variants following Evusheld admininistration, to characterise the PK of AZD3152 and Evusheld in serum and to evaluate the ADA responses to AZD3152 and AZD7442 in serum	• FPCD: Q4 2022 • LPCD: Q4 2023 • Data readout: Q2 2024 • Primary Endpoint met
Phase I **LITTLE DIPPER** **NCT05872958**	Healthy adult participants; age 18 to 55 years	96	• Phase I, double-blind, placebo-controlled, multi-centre, dose exploration trial • to evaluate the safety and PK of AZD3152 in healthy adult participants across different dose levels and routes of administration • participants randomised in a 10:2 ratio to receive either AZD3152 or placebo administered i.m. or i.v. across 5 fixed-dose cohorts	• Primary endpoint: to evaluate the safety of i.m. or i.v. administration of AZD315 and to characterise the PK of AZD3152 in serum after a single i.m. or i.v. dose • Secondary endpoint: to evaluate ADA responses to AZD3152	• FPCD: Q2 2023 • LPCD: Q3 2023 • Data readout: Q4 2023 • Primary endpoint met

As of 29 July 2025.
Appendix: Glossary.



BioPharmaceuticals: early-stage development

AstraZeneca

opemalirsen (AZD2373, APOL1)

Chronic kidney disease

Trial	Population	Patients	Design	Endpoints	Status
Phase IIb **APPRECIATE** **NCT06824987** **Partnered (Ionis Pharmaceuticals, Inc.)**	Participants diagnosed with APOL1 mediated kidney disease (AMKD), proteinuria, 18-65 years of age	96	• Randomised, multi-centre, double-blind trial in US and UK followed by OLE • Arm 1: opemalirsen dose A • Arm 2: opemalirsen dose B • Arm 3: placebo	• Primary endpoint: Dose-response effect of AZD2373 on placebo corrected percentage change in uACR from baseline to Week 30 • Secondary endpoints: Safety and tolerability, proportion of patients achieving a 45% or greater reduction in uACR	• FPCD: Q2 2025 • Data anticipated: >2026
Phase I **NCT04269031**	Healthy volunteers	30	• SAD dose escalation in 6 cohorts with 6 volunteers receiving opemalirsen and 2 volunteers receiving placebo in each cohort • Arm 1: opemalirsen s.c. • Arm 2: placebo s.c. • US only	• Primary endpoints: safety and tolerability • Secondary endpoint: PK parameters	• FPCD: Q1 2020 • LPCD: Q3 2021 • Data readout: Q3 2022
Phase I **NCT05351047**	Healthy volunteers	24	• MAD dose escalation in 3 cohorts with 6 volunteers per cohort receiving opemalirsen and 2 volunteers per cohort receiving placebo • Arm 1: opemalirsen s.c. • Arm 2: placebo s.c. • US only	• Primary endpoints: safety and tolerability • Secondary endpoints: PK parameters, effect of s.c. MAD administrations of AZD2373 on plasma concentrations of APOL1 protein and APOL1 G0, G1, G2 allele genotype status in trial participants	• FPCD: Q2 2022 • LPCD: Q1 2023 • Data readout: Q4 2023

As of 29 July 2025.
Appendix: Glossary.

AZD0233 (oral CX3CR1)

Dilated cardiomyopathy

Trial	Population	Patients	Design	Endpoints	Status
Phase I **NCT06381466**	Healthy volunteers	96	• Randomised, SAD/MAD dose escalating trial	• Primary endpoints: safety and tolerability • Secondary endpoints: PK parameters	• FPCD: Q2 2024 • Data anticipated: H2 2025

As of 29 July 2025.
Appendix: Glossary.

AZD1613 (PAPPA-1 MAb)

ADPKD

Trial	Population	Patients	Design	Endpoints	Status
Phase I **NCT06995820**	Healthy volunteers	136	• Randomised, single-blind, placebo-controlled single and multiple ascending dose	• Primary endpoint: safety and tolerability • Secondary endpoints: PK parameters, changes in plasma PD biomarkers, changes in plasma PD biomarkers'	• FPCD: Q2 2025 • Data anticipated: H2 2026

As of 29 July 2025.
Appendix: Glossary.

AZD1705 (Angptl3 inhibitor)

Dyslipidaemia

Trial	Population	Patients	Design	Endpoints	Status
Phase I **NCT06238466**	Dyslipidaemia	112	• Part A: single dose of AZD1705 with an in-clinic period of 3 days followed by an outpatient follow-up period of approximately 16 weeks • Part B: 2 doses of AZD1705 given 28 days apart with an in-clinic period followed by an outpatient follow-up period of approximately 20 weeks	• Primary endpoints: AEs and SAEs • Secondary endpoints: AUCinf, AUClast, Cmax, Ae, fe, CLR, LDL-C, ApoB, triglycerides and target plasma protein	• FPCD: Q1 2024 • LPCD: Q2 2025 • Data anticipated: H1 2026

As of 29 July 2025.
Appendix: Glossary.

AZD2389 (anti-fibrotic mechanism)

MASH

Trial	Population	Patients	Design	Endpoints	Status
Phase II **BORANA** **NCT06750276**	Participants with liver fibrosis and compensated cirrhosis	36	• Randomised, single-blind, placebo-controlled trial	• Primary endpoints: safety and tolerability	• FPCD: Q4 2024 • Data anticipated: H2 2025
Phase I **NCT06138795**	Healthy volunteers	126	• Randomised, placebo-controlled SAD/MAD trial	• Primary endpoints: safety and tolerability	• FPCD: Q4 2023 • Data anticipated: H2 2025
Phase I **NCT06812780** -	Healthy volunteers or participants with hepatic impairment	36	• Multi-centre, single-dose, non-randomised, open-label, parallel-group trial	• Primary endpoint: PK parameters • Secondary endpoints: safety and tolerability	• FPCD: Q1 2025 • Data anticipated: H2 2025
Phase I **NCT06846528**	Healthy volunteers	16	• Open-label, fixed-sequence trial • AZD2389 • AZD2389 + itraconazole	• Primary endpoints: PK parameters, safety and tolerability	• FPCD: Q1 2025 • LPCD: Q2 2025 • Data anticipated: H2 2025
Phase I **06974565**	Healthy Volunteers	24	• Open-label, randomized, single dose, 2-way crossover	• Pharmacokinetics, safety and tolerability	• FPCD: Q2 2025 • Data anticipated: H2 2025
Phase I **06973005**	Healthy Volunteers	8	• Open label, fixed sequence, 3 period	• Pharmacokinetics, safety and tolerability	• FPCD: Q2 2025 • Data anticipated: H2 2025

As of 29 July 2025.
Appendix: Glossary.

AZD2693 (PNPLA3 ASO)

MASH

Trial	Population	Patients	Design	Endpoints	Status
Phase IIb **FORTUNA** **NCT05809934**	MASH with fibrosis	180	• Randomised, double-blind, placebo-controlled, multi-centre trial • Arm 1: AZD2693 s.c. dose 1 • Arm 2: AZD2693 s.c. dose 2 • Arm 3: placebo s.c.	• Primary endpoints: efficacy, safety and tolerability	• FPCD: Q2 2023 • LPCD: Q3 2024 • Data anticipated: H1 2026
Phase I **NCT04483947**	NASH/NAFLD F0-F3	74	• MAD with 4 cohorts receiving AZD2693 and placebo in each cohort • Arm 1: AZD2693 s.c. • Arm 2: placebo s.c. • US only	• Primary endpoints: safety and tolerability • Secondary endpoint: PK parameters	• FPCD: Q2 2021 • LPCD: Q3 2023 • Data readout: Q2 2024
Phase I **NCT05107336**	Healthy volunteers	44	• MAD with 4 cohorts receiving AZD2693 and placebo in each cohort • Arm 1: AZD2693 s.c. • Arm 2: placebo s.c. • JP only	• Primary endpoints: safety and tolerability • Secondary endpoint: PK parameters	• FPCD: Q4 2021 • LPCD: Q4 2022 • Data readout: Q4 2023
Phase I **NCT05919069**	Hepatic impairment	32	• Single-dose, non-randomised, open-label, parallel group trial • US only	• Primary endpoints: safety, tolerability and PK parameters	• FPCD: Q3 2023 • LPCD: Q2 2024 • Data readout: Q4 2024

As of 29 July 2025.
Appendix: Glossary.

AZD3427 (relaxin)

Heart failure

Trial	Population	Patients	Design	Endpoints	Status
Phase II **Re-PHiRE** **NCT05737940**	HF and pulmonary hypertension due to left heart disease	260	• Randomised, double-blind, placebo-controlled, multi-centre trial • Arm 1: AZD3427 (high dose) • Arm 2: AZD3427 (medium dose) • Arm 3: AZD3427 (low dose) • Arm 4: placebo	• Primary endpoint: change in PVR from baseline to Week 25 vs. placebo as measured by right heart catheterisation	• FPCD: Q2 2023 • Data anticipated: H2 2025
Phase Ib **RE-PERFUSE** **NCT06611423**	HFrEF patients with mild renal impairment	12	• Eligible participants randomised equally • Arm 1: i.v. saline placebo followed by s.c. AZD3427 • Arm 2: i.v. saline placebo followed by s.c. AZD3427 placebo • Arm 3: i.v. dopamine diluted in saline followed by s.c. AZD3427 • Arm 4: i.v. dopamine diluted in saline followed by s.c. AZD3427 placebo	• Primary endpoint: volumetric fraction of the renal cortex with increased perfusion from baseline to Day 8 compared to placebo as measured using PET	• FPCD: Q4 2024 • Data anticipated: H2 2025

As of 29 July 2025.
Appendix: Glossary.

AZD4144 (inflammation modulator)

Cardiorenal disease

Trial	Population	Patients	Design	Endpoints	Status
Phase I **NCT06122714**	Healthy participants	95	• Randomised, single-blind, placebo-controlled, SAD/MAD sequential group trial	• Primary endpoints: safety and tolerability • Secondary endpoints: PK parameters	• FPCD: Q4 2023 • LPCD: Q4 2024 • Data readout: Q1 2025
Phase I **NCT06491550**	Healthy participants	92	• Randomised, single-blind, placebo-controlled, SAD/MAD sequential group trial	• Primary endpoints: safety and tolerability • Secondary endpoints: PK parameters	• FPCD: Q3 2024 • LPCD: Q2 2025 • Data anticipated: H2 2025
Phase I **NCT06693765**	Participants with renal impairment, end-stage kidney disease and healthy volunteers	41	• Single-dose, non-randomised, open-label, parallel-group trial	• Primary endpoints: safety, tolerability and PK parameters	• FPCD: Q4 2024 • LPCD: Q2 2025 • Data anticipated: H2 2025
Phase I **NCT06675175**	Participants with established ASCVD	28	• Randomised, double-blind, placebo-controlled, parallel group trial	• Primary endpoints: safety, tolerability and PD parameters • Secondary endpoints: PK and PD parameters	• FPCD: Q1 2025 • Data anticipated: H2 2025
Phase I **NCT06948006**	Healthy participants	32	• Open-label, randomised, single-dose, crossover study	• Primary endpoints: PK and safety	• FPCD: Q2 2025 • LPCD: Q2 2025 • Data anticipated: H2 2025
Phase I **NCT06925854**	Healthy participants	12	• Open-label, 2-period, 2-sequence cross-over trial • Treatment A: single dose of rosuvastatin • Treatment B: single dose of rosuvastatin in combination with AZD4144 • Participants will be randomized 1:1 ratio to receive treatment sequence AB or BA.	• Primary endpoints: PK and safety	• FPCD: Q2 2025 • LPCD: Q2 2025 • Data anticipated: H2 2025
Phase I **NCT06942923**	Healthy participants with obesity	28	• Placebo-controlled, parallel group study	• Primary endpoints: safety, tolerability and PD parameters • Secondary endpoints: PK and PD parameters	• FPCD: Q2 2025 • Data anticipated: H2 2025

As of 29 July 2025.
Appendix: Glossary.

AZD4248 (oral NNMT)

CKD

Trial	Population	Patients	Design	Endpoints	Status
Phase I **NCT07024823**	Healthy volunteers	164	• Randomised, single-blind, placebo-controlled • Part A: SAD in healthy volunteers • Part B: MAD in healthy volunteers • Part C: multiple dosing DKD • Part D: observational cohort	• Primary endpoints: Safety and tolerability measures • Secondary endpoints: PK parameters	• FPCD: Q2 2025 • Data anticipated: H1 2026

As of 29 July 2025.
Appendix: Glossary.

AZD4954 (Lp(a) inhibitor)

Dyslipidaemia

Trial	Population	Patients	Design	Endpoints	Status
Phase I **NCT06980428**	Healthy volunteers	120	• Randomised, placebo-controlled SAD/MAD trial	• Primary endpoints: safety and tolerability	• FPCD: Q2 2025 • Data anticipated: H2 2026

As of 29 July 2025.
Appendix: Glossary.

AZD5004 (oral GLP-1 RA)

Type 2 diabetes, obesity

Trial	Population	Patients	Design	Endpoints	Status
Phase IIb **SOLSTICE** **NCT06579105**	Type 2 diabetes	406	• Arm 1: AZD5004 tablet • Arm 2: AZD5004 tablet • Arm 3: AZD5004 tablet • Arm 4: AZD5004 tablet • Arm 5: AZD5004 tablet • Arm 6: AZD5004 tablet • Arm 7: active comparator semaglutide tablet • Arm 8: placebo matching AZD5004 tablet	• Primary endpoint: change in HbA1c from baseline at 26 weeks • Secondary endpoints: change in fasting glucose from baseline, proportion of participants achieving HbA1c ≤6.5% and baseline HbA1c ≥7% and achieving <7.0% and percent change in body weight from baseline	• FPCD: Q4 2024 • LPCD: Q2 2025 • Data anticipated: H1 2026
Phase IIb **VISTA** **NCT06579092**	Obesity or overweight who have at least one weight-related comorbidity	310	• Arm 1: AZD5004 tablet • Arm 2: AZD5004 tablet • Arm 3: AZD5004 tablet • Arm 4: AZD5004 tablet • Arm 5: AZD5004 tablet • Arm 6: placebo matching AZD5004 tablet	• Primary endpoints: percent change in body weight from baseline at 26 weeks and proportion of participants with weight loss ≥5% from baseline weight at 26 weeks • Secondary endpoints: percent change in body weight from baseline at 36 weeks, proportion of participants with weight loss ≥5% and absolute change from baseline in body weight at 26 and 36 weeks	• FPCD: Q4 2024 • Data anticipated: H1 2026

As of 29 July 2025.
Appendix: Glossary.

AZD5004 (oral GLP-1 RA)

Type 2 diabetes, obesity

Trial	Population	Patients	Design	Endpoints	Status
Phase I **NCT06555822**	Healthy volunteers	31	• Part A – Arm 1: AZD5004 oral tablet • Part A – Arm 2: placebo oral tablet • Part B: single dose, open label crossover	• Primary endpoints (Part A): Safety and tolerability • Secondary endpoints (Part A): PK and PD parameters • Primary endpoint (Part B): PK parameters • Secondary endpoints (Part B): Safety and tolerability	• FPCD: Q3 2024 • LPCD: Q1 2025 • Data readout: Q2 2025
Phase I **NCT06703658**	Healthy volunteers or participants with type 2 diabetes mellitus	35	• SAD: 3 cohorts to receive AZD5004 or placebo tablet • MAD: 1 cohort to receive AZD5004 or placebo tablet • Japan only	• Primary endpoints: safety and tolerability • Secondary endpoints: PK and PD parameters	• FPCD: Q4 2024 • LPCD: Q1 2025 • Data anticipated: H2 2025
Phase I **NCT06742762**	Healthy volunteers or participants with renal impairment	16	• Multi-centre, single-dose, non-randomised, open-label, parallel-group trial, Single oral dose of AZD5004 •	• Primary endpoint: PK parameters • Secondary endpoints: safety and tolerability	• FPCD: Q1 2025 • Data anticipated: H2 2025
Phase I **NCT06813781**	Healthy volunteers or participants with hepatic impairment	32	• Multi-centre, single-dose, non-randomised, open-label, parallel-group trial, Single oral dose of AZD5004 •	• Primary endpoint: PK parameters • Secondary endpoints: safety and tolerability	• FPCD: Q1 2025 • Data anticipated: H1 2026
Phase I **NCT06857695**	Healthy volunteers	8	• Part 1: A single dose of AZD5004 film-coated tablet and a single dose of AZD5004 solution for infusion • Part 2: A single dose of AZD5004 oral solution	• Part 1: absolute bioavailability • Part 2: amount of AZD5004 excreted	• FPCD: Q1 2025 • Data anticipated: H2 2025
Phase I **NCT06988553** **Partnered (Eccogene)**	Participants with overweight/obesity with/without T2D	45	• Randomized, parallel group, double-blind trial • AZD5004 or placebo tablet • China only	• Primary endpoints: safety and tolerability	• FPCD: Q2 2025 • Data anticipated: H1 2026

As of 29 July 2025.
Appendix: Glossary.

AZD5004 (oral GLP-1 RA)

Type 2 diabetes, obesity

Trial	Population	Patients	Design	Endpoints	Status
Phase I **NCT06996886**	Healthy volunteers	16	• Open-label, randomized, 4-period, 4-treatment, single-dose crossover trial • Different formulations as single dose of AZD5004	• Primary endpoint: PK profile (relative bioavailability) • Secondary endpoints: PK profile (food effect) • Safety endpoints: safety and tolerability	• FPCD: Q2 2025 • Data anticipated: H1 2026
Phase I **NCT06948747**	Healthy volunteers	48	• Open-label, fixed-sequence, single-centre trial • Part A: AZD5004, Rosuvastatin and Erythromycin tablets • Part B: AZD5004, Atorvastatin and Simvastatin tablets • Part C: AZD5004 and Repaglinide tablets	• Part A, B and C: • Primary endpoints: PK profile • Safety endpoints: safety and tolerability	• FPCD: Q2 2025 • Data anticipated: H2 2025
Phase I **NCT06942936**	Healthy volunteers	48	• Open-label, fixed-sequence, two-part study • Part A: AZD5004 tablet and Itraconazole capsule • Part B: AZD5004 and estradiol/levonorgestrel tablets	• Primary endpoint: PK profile • Safety endpoints: safety and tolerability	• FPCD: Q2 2025 • Data anticipated: H2 2026

As of 29 July 2025.
Appendix: Glossary.

AZD5462 (oral relaxin)

Heart failure

Trial	Population	Patients	Design	Endpoints	Status
Phase IIb **LUMINARA** **NCT06299826**	Stable patients with chronic heart failure	360	• Two cohort, randomised, double-blind, placebo-controlled, multi-centre trial • Arm 1: AZD5462 (high dose) • Arm 2: AZD5462 (medium dose) • Arm 3: AZD5462 (low dose) • Arm 4: placebo	• Primary endpoint: change in heart function from baseline to Week 25 compared to placebo	• FPCD: Q3 2024 • Data anticipated: H2 2026
Phase Ib **AURORA** **NCT06639087**	Stable patients with heart failure and moderately impaired renal function	8	• Randomised, double-blind, placebo-controlled, multi-centre mechanistic trial • Arm 1: AZD5462 + dapagliflozin • Arm 2: placebo + dapagliflozin	• Primary endpoint: change in fractional excretion of sodium from baseline to Day 1	• FPCD: Q4 2024 • LPCD: Q2 2025 • Data anticipated: H1 2026
Phase I **GLITTER** **NCT06661733**	Participants with Severe Renal Impairment and participants Normal Renal Function	16	• Single centre, non-randomised, open-label, parallel group trial • Cohort 1: AZD5462 • Cohort 2: AZD5462	• Primary endpoints: PK parameters, safety and tolerability	• FPCD: Q4 2024 • LPCD: Q4 2024 • Data anticipated: H2 2025
Phase I **PHOTON** **NCT06989983**	Healthy volunteers	8	• Open-label, two-part sequential human ADME trial	• Primary endpoints: mass balance recovery, absorption, metabolism, excretion of [14C]AZD5462 and absolute bioavailability of AZD5462 • Secondary endpoints: safety and tolerability	• FPCD: Q2 2025 • Data anticipated: H1 2026

As of 29 July 2025.
Appendix: Glossary.

AZD6234 (selective amylin agonist)

Obesity with related co-morbidities

Trial	Population	Patients	Design	Endpoints	Status
Phase II **APRICUS** **NCT06595238**	Participants living with obesity or overweight with co-morbidity	231	• Randomised, double-blind, placebo-controlled trial	• Primary endpoints: percent change in body weight from baseline to Week 26 and weight loss ≥5% from baseline weight to Week 26	• FPCD: Q4 2024 • Data anticipated: H2 2025
Phase II **ARAY** **NCT06851858**	adults with overweight or obesity and type 2 diabetes on stable GLP-1 RA therapy	64	• Randomised, parallel-group, double-blind, placebo-controlled trial •	• Primary endpoints: Percent change in body weight from baseline at Study Week 2; Weight loss ≥ 5% from baseline at Study Week 26 • Secondary endpoints: Weight loss, HbA1c, PK measures	• FPCD: Q2 2025 • Data anticipated: H1 2026
Phase I/II **NCT07017179**	Chinese participants with obesity/overweight	18	• study will comprise 3 periods totalling up to approximately 23 weeks	• To assess the safety and tolerability of repeated subcutaneous(SC) doses of AZD6234 compared to placebo.	• FPCD: Q2 2022 • Data anticipated: H1 2026
Phase I **NCT05511025**	Healthy participants who are overweight or obese	64	• SAD trial	• Primary endpoint: safety	• FPCD: Q4 2022 • Data readout: Q1 2024
Phase I **NCT06132841**	Overweight or obese participants	142	• Randomised, single-blind, placebo-controlled trial with repeated doses of AZD6234 or placebo via s.c. injection	• Primary endpoints: safety and tolerability of repeat doses	• FPCD: Q4 2023 • Data anticipated: H1 2026
Phase I **NCT06845813**	Participants include those with end-stage renal disease (ESRD) on intermittent haemodialysis (HD), severe renal impairment not on dialysis, and optional groups for moderate and mild renal impairment.	48	• Phase I multicentre, single-dose, non-randomised, open-label, parallel-group study aims to examine the pharmacokinetics, safety, and tolerability of AZD6234 in both male and female participants.	• compare the plasma PK of a single SC dose of AZD6234 in participants with ESRD on HD, severe renal impairment (not on dialysis), moderate (optional), and mild (optional) renal impairment to those with normal renal function	• FPCD: Q1 2025 • Data anticipated: H1 2026
Phase I **NCT07013643**	healthy females of childbearing and non-childbearing potential	25	• open-label, single-sequence, single-cohort study	• To assess the effect of multiple doses of AZD6234 on the PK of single doses of combined oral contraceptive EE/LEVO.	• FPCD: Q3 2025 • Data anticipated: H1 2026

As of 29 July 2025.
Appendix: Glossary.

AZD9550 (GLP-1-glucagon agonist)

MASH

Trial	Population	Patients	Design	Endpoints	Status
Phase I **NCT05848440**	Healthy volunteers	64	• SAD trial	• Primary endpoints: safety and tolerability	• FPCD: Q2 2023 • LPCD: Q4 2023 • Data readout: Q2 2024
Phase I **CONTEMPO** **NCT06151964**	Overweight and obese participants with T2DM	90	• Randomised, single-blind, placebo-controlled, MAD trial with 4 parts (A to D) • Part A: multiple repeat doses of AZD9550 or placebo given as 4 QW s.c. doses for 4 weeks to 2 sequential cohorts evaluating 2 low dose levels of AZD9550 or placebo • Part B: QW up-titration over 5 doses of AZD9550 or placebo • Part C: bi-weekly/monthly up-titration of AZD9550 or placebo for 24 weeks • Part D: bi-weekly/monthly up-titration of AZD9550 or placebo for 24 weeks (Japan only)	• Primary endpoints: safety, tolerability and PK parameters	• FPCD: Q4 2023 • Data anticipated: H2 2026

As of 29 July 2025.
Appendix: Glossary.

AZD9550+AZD6234 (GLP-1-glucagon agonist + selective amylin agonist)

Obesity

Trial	Population	Patients	Design	Endpoints	Status
Phase IIb **ASCEND** **NCT06862791**	Adults who are living with obesity or overweight with at least one of the following weight-related co-morbidities: hypertension, dyslipidemia or obstructive sleep apnoea	360	• Randomised, parallel-group, double-blind, placebo-controlled, multi-centre, reduced factorial design • IMP injected subcutaneous, once weekly • Arm 1: AZD9550 low dose + AZD6234 low dose or placebos • Arm 2: AZD9550 medium dose + AZD6234 medium dose or placebos • Arm 3: AZD9550 high dose + AZD6234 high dose or placebos • Arm 4: AZD9550 low dose + AZD6234 medium dose or placebos • Arm 5: AZD9550 medium dose + AZD6234 low dose or placebos • Arm 6: AZD9550 high dose + AZD6234 medium dose or placebos • Arm 7: AZD9550 medium dose + AZD6234 high dose or placebos • Arm 8: AZD9550 high dose or placebo • Arm 9: AZD6234 high dose or placebo	• Primary endpoints: percent change in body weight from baseline after 36 weeks of treatment, weight loss ≥ 5% from baseline after 36 weeks of treatment • Secondary endpoints: absolute body weight change, weight loss ≥5%/10%/15% from baseline, ADA incidence/prevalence/titres	• FPCD: Q1 2025 • Data anticipated: H2 2026

As of 29 July 2025.
Appendix: Glossary.

atuliflapon (FLAP inhibitor)

Asthma

Trial	Population	Patients	Design	Endpoints	Status
Phase IIa **FLASH** **NCT05251259**	Patients with moderate-to-severe uncontrolled asthma	666	• Randomised, placebo-controlled, double-blind, multi-centre trial with a lead-in PK cohort • PK cohort • Arm 1: atuliflapon • Arm 2: placebo • Part 1 • Arm 1: atuliflapon • Arm 2: placebo • Global trial	• Primary endpoint: time to first CompEx asthma event	• FPCD: Q2 2022 • Data anticipated: H1 2026

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Appendix: Glossary.

AZD0120 (GC012F, autologous anti-CD19 and anti-BCMA CAR-T)

SLE

Trial	Population	Patients	Design	Endpoints	Status
Phase Ib/II NCT06897930	Refractory systemic lupus erythematosus (SLE)	24	• Single-arm, open-label, multi-center trial	• Primary endpoints (Phase I): safety and tolerability, determination of recommended dose for expansion phase • Secondary endpoints (Phase I): SRI-4, DORIS, LLDAS, BICLA, time from infusion to disease flare, PK parameters, LN-specific responses, disease related biomarker assessments, AZD0120 immunogenicity, RCL presence	• Data anticipated: >2026 • Initiating
Phase I/II NCT06530849	Refractory systemic lupus erythematosus	21	• Single-arm, open label, multi-centre trial	• Primary endpoint (Phase I): safety at 28 days • Primary endpoint (Phase II): efficacy (SRI-4 response) at Week 48	• FPCD: Q3 2024 • Data anticipated: >2026

As of 29 July 2025.
Appendix: Glossary.

AZD1163 (anti-PAD2/4 bispecific antibody)

Rheumatoid arthritis

Trial	Population	Patients	Design	Endpoints	Status
Phase I **NCT06103877**	Healthy volunteers	99	• Randomised, double-blind, placebo-controlled SAD/MAD trial • Part 1 (SAD): 10 cohorts with 8 i.v. administered dose levels and 1 s.c. administered dose level of AZD1163 • Part 2 (MAD): 4 s.c. dose levels of AZD1163	• Primary endpoint: incidence of AEs • Secondary endpoint: PK parameters	• FPCD: Q4 2023 • Data anticipated: H1 2026

As of 29 July 2025.
Appendix: Glossary.

AZD4604 (inhaled JAK-1 inhibitor)

Asthma

Trial	Population	Patients	Design	Endpoints	Status
Phase IIa **AJAX** **NCT06020014**	Moderate-to-severe asthma uncontrolled on medium-to high-dose ICS-LABA	320	• Multi-centre, randomised, placebo-controlled, double-blind, parallel-group trial • Arm 1: AZD4604 • Arm 2: placebo	• Primary endpoint: time to first CompEx asthma event • Secondary endpoints: Pre-BD FEV1, CAAT, ACQ-6, average morning and average evening PEF, daily asthma symptom score, time to first CompEx acute worsening event, CompEx event rate and CompEx acute worsening event rate	• FPCD: Q4 2023 • Data anticipated: H1 2026
Phase IIa **ARTEMISIA** **NCT06435273**	Adult patients with moderate-to-severe asthma receiving treatment with medium-to-high dose ICS-LABA	48	• Multi-centre, randomised, placebo-controlled, double-blind, parallel-group trial • Arm 1: AZD4604 • Arm 2: placebo	• Primary endpoint: gene expression in airway epithelial cells • Secondary endpoints: STAT phosphorylation and cellular pathology	• FPCD: Q3 2024 • Data anticipated: H1 2026
Phase Ib **ATALANTA** **NCT06732882**	Adults With Mild Asthma	28	• Single bilnd, multi-center, randomised, placebo-controlled, parallel-group trial via the Turbuhaler and Genuair devices • Arm 1: Genuair 1400 ug BID AZD4604 • Arm 2: Turbuhaler 1400 ug BID AZD4604 • Arm 3: Turbuhaler 150 ug BID AZD4604 • Arm 4: Genuair placebo • Arm 5: Turbuhaler Placebo	• Primary endpoints: PK parameters • Secondary endpoints: PD parameters, FeNO •	• FPCD: Q1 2025 • Data anticipated: H2 2025
Phase I **NCT04769869**	Healthy volunteers and patients with mild asthma	137	• SAD/MAD/POM trial • Part 1 SAD • Part 2 MAD • Part 3 POM • UK only	• Primary endpoints: safety and tolerability • Secondary endpoints: PK parameters and FENO	• FPCD: Q4 2021 • LPCD: Q1 2023 • Data readout: Q1 2023
Phase I **NCT06519968**	Healthy volunteers	56	• Part 1a: SAD cohorts in healthy Japanese participants • Part 1b: multiple dose cohort in healthy Japanese participants • Part 2a: SAD cohort in healthy Chinese participants • Part 2b: multiple dose cohort in healthy Chinese participants	• Primary endpoints: safety and tolerability • Secondary endpoints: PK parameters	• FPCD: Q3 2024 • LPCD: Q4 2024 • Data readout: Q1 2025

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Appendix: Glossary.

AZD5492 (CD20 TITAN TCE)

SLE

Trial	Population	Patients	Design	Endpoints	Status
Phase I TITAN NCT06916806	Systemic lupus erythematosus (SLE) or Idiopathic inflammatory myopathies (IIM)	50	• Open-label, multii-centre • Part 1: Single ascending dose with AZD5492 • Part 2: Step-up dosing with AZD5492	• Primary endpoints: safety and tolerability • Secondary endpoints: PK parameters	• Data anticipated: >2026

As of 29 July 2025.
Appendix: Glossary.

AZD6793 (IRAK4)

Inflammatory diseases

Trial	Population	Patients	Design	Endpoints	Status
Phase II **PRESTO** **NCT07082738**	moderate to very severe COPD	1160	• Randomised, double-blind, placebo controlled 4 arm study • Dose 1 AZD6793 • Dose 2 AZD6793 • Dose 3 AZD6793 • Placebo	• Primary endpoint: annualised rate of moderate or severe COPD exacerbations • Secondary endpoints: time to first exacerbations, annualised rate of severe exacerbations, CompEx, pre-BD FEV1, post-BD FEV1, BCSS, CAT, SGRQ	• *Initiating* • Data anticipated: >2026
Phase I **NCT05662033**	Healthy volunteers	133	• Single-blind, randomised, placebo-controlled trial	• Primary endpoints: safety and tolerability • Secondary endpoint: PK parameters	• FPCD: Q4 2022 • LPCD: Q4 2024 • Data anticipated: H2 2025
Phase I **NCT06368440**	Healthy volunteers	40	• Single-blind, randomised, placebo-controlled trial • Japanese and Chinese healthy participants	• Primary endpoint: safety • Secondary endpoints: PK parameters	• FPCD: Q2 2024 • LPCD: Q4 2024 • Data readout: Q2 2025
Phase I **NCT06494644**	Healthy participants	17	• A single-group trial with a duration of up to 8 weeks (maximum of 53 days) including Screening, Period 1, Period 2, Period 3 and Follow-up to assess the pharmacokinetics of AZD6793 when administered alone and in combination with itraconazole in healthy participants	• Primary endpoint: PK parameters (Cmax, AUC, CL/F, t1/2, tmax, Vz/F, RAUC) • Secondary endpoint: safety	• FPCD: Q3 2024 • LPCD: Q4 2024 • Data readout: Q2 2025

As of 29 July 2025.
Appendix: Glossary.

AZD6912 (siRNA)

Rheumatoid arthritis

Trial	Population	Patients	Design	Endpoints	Status
Phase I **NCT06115967**	Healthy volunteers	64	• Randomised, double-blind, placebo-controlled SAD trial • 8 cohorts with s.c. administered dose level of AZD6912	• Primary endpoint: incidence of AEs • Secondary endpoint: PK parameters	• FPCD: Q4 2023 • Data anticipated: >2026

As of 29 July 2025.
Appendix: Glossary.

AZD7798 (humanised mAb)

Crohn's disease

Trial	Population	Patients	Design	Endpoints	Status
Phase IIa **AMALTHEA** **NCT06450197**	Moderate to severe Crohn's disease	192	• Randomised, double-blind, placebo-controlled trial • Arm 1: AZD7798 • Arm 2: placebo	• Primary endpoint: Crohn's Disease Activity Index (CDAI) remission • Secondary endpoints: endoscopic response, endoscopic remission, endoscopic score change from baseline, CDAI response, CDAI score change from baseline, symptomatic remission, PK parameters and ADA	• FPCD: Q4 2024 • Data anticipated: H1 2026
Phase II **CALLISTO** **NCT06681324**	Patients with active ileal Crohn's disease and an ileostomy	30	• A Participant- and Investigator-blind, Randomized, Placebo-controlled Phase II Study Arm 1: AZD7798 • Arm 2: Placebo	• Primary endpoint: safety • Secondary endpoints: Simple Endoscopic Score for Crohn's Disease (SES-CD) , endoscopic response and remission, PK parameters, ADA	• FPCD: Q1 2025 • Data anticipated: H1 2026
Phase I **NCT05452304**	Global, Japanese and Chinese healthy volunteers	144	• SAD, repeating dose trial • Arm 1: AZD7798 • Arm 2: placebo • s.c. and i.v. administration • UK only	• Primary endpoints: safety and tolerability • Secondary endpoints: PK parameters and immunogenicity	• FPCD: Q3 2022 • LPCD: Q3 2024 • Data readout: Q4 2023

As of 29 July 2025.
Appendix: Glossary.

AZD8630 (inhaled TSLP)

Asthma

Trial	Population	Patients	Design	Endpoints	Status
Phase II **LEVANTE** **NCT06529419** **Partnered (AMGEN)**	Adults with uncontrolled asthma at risk of exacerbations	516	• Randomised, placebo-controlled, double-blind, dose range-finding, multi-centre trial • Arm 1: AZD8630 Dose A • Arm 2: AZD8630 Dose B • Arm 3: AZD8630 Dose C • Arm 4: placebo	• Primary endpoint: time to first CompEx asthma event • Secondary endpoints: change from baseline in pre-bronchodilator forced expiratory volume in 1 second and safety and tolerability	• FPCD: Q3 2024 • Data anticipated: H1 2026
Phase I **NCT05110976** **Partnered (AMGEN)**	Healthy volunteers and patients with asthma	232	• SAD and MAD trial	• Primary endpoints: safety and tolerability • Secondary endpoints: PK parameters and FENO	• FPCD: Q1 2022 • LPCD: Q3 2023 • Data readout: Q4 2023 • Primary and Secondary endpoints met
Phase I **NCT06531811** **Partnered (AMGEN)**	Healthy volunteers	28	• Randomised, open-label, 2-treatment, 2-period trial	• Primary endpoints: safety and tolerability • Secondary endpoint: PK parameters	• FPCD: Q3 2024 • LPCD: Q3 2024 • Data readout: Q2 2025
Phase I **NCT06795906** **Partnered (Amgen)**	Adults with asthma on medium-to-high dose inhaled corticosteroids and long-acting beta-agonists	24	• Randomised, placebo-controlled, double-blind, parallel design	• Primary endpoints: safety and tolerability, pharmacokinetic parameter • Secondary endpoint : change from baseline in FeNO at weeks 1 and 2	• FPCD: Q1 2025 • LPCD: Q2 2025 • Data anticipated: H2 2025

As of 29 July 2025.
Appendix: Glossary.

AZD8965 (arginase enzyme inhibitor)

IPF

Trial	Population	Patients	Design	Endpoints	Status
Phase I **NCT06502379**	Healthy volunteers	163	• Randomised, single-blind, SAD/MAD, placebo-controlled, AZD8965/placebo administered orally • PART 1: SAD cohorts • PART 2: MAD cohorts • PART 3a: Japanese and Chinese participants SAD cohorts • PART 3b: Japanese and Chinese participants SMAD cohorts • PART 4: food effect cohort	• Primary endpoints (Part 1, 2, 3): safety and tolerability measures • Primary endpoint (Part 4): PK parameters • Secondary endpoint (Part 1, 2, 3): PK parameters • Secondary endpoints (Part 4): safety and tolerability measures under fasted and fed condition	• FPCD: Q3 2024 • Data anticipated: H2 2025

As of 29 July 2025.
Appendix: Glossary.

mRNA VLP vaccine

COVID-19

Trial	Population	Patients	Design	Endpoints	Status
Phase I **ARTEMIS-C** **NCT06147063**	Healthy volunteers ≥18+ with history of a SARS-CoV-2 infection and/or prior completion of primary series/booster vaccination at least 6 months prior to trial start	240	• Arm 1: dose 1 via i.m. injection AZD9838 in 18-64-year-olds • Arm 2: dose 2 via i.m. injection AZD9838 in 18-64-year-olds • Arm 3: i.m. dose of licensed mRNA vaccine in 18-64-year-olds • Arm 4: dose 1 via i.m. injection AZD6563 in 18-64-year-olds • Arm 5: dose 2 via i.m. injection AZD6563 in 18-64-year-olds • Arm 6: dose 1 via i.m. injection in 65+ year olds • Arm 7: dose 2 via i.m. injection in 65+ year olds • Arm 8: i.m dose of licensed mRNA vaccine in 65+ year olds	• Primary endpoints: safety as measured by AEs, ARs, SAEs, MAAEs, AESIs, GMTs of strain neutralising antibodies and GMFRs of strain neutralising antibodies • Secondary endpoints: nAb responses to the SARS-CoV2 ancesteral strain, Omicron BA.4/5, and Omicron XBB.1.5 in serum	• FPCD: Q4 2023

As of 29 July 2025.
Appendix: Glossary.

AZD0292 (Psl-PcrV N3Y-bispecific mAb)

Bronchiectasis

Trial	Population	Patients	Design	Endpoints	Status
Phase I NCT06311760	Healthy volunteers	32	• Randomised, single-blind, placebo-controlled trial • Arm 1: AZD0292 Dose 1 administered via i.v. infusion • Arm 2: AZD0292 Dose 2 administered via i.v. infusion • Arm 3: AZD0292 Dose 3 administered via i.v. infusion • Arm 4: AZD0292 Dose 4 administered via i.v. infusion • Arm 5: placebo administered via i.v. infusion	• Primary endpoints: AEs and participants with AESI • Secondary endpoints: Cmax, AUClast, AUCinfinity and ADA	• FPCD: Q2 2024 • LPCD: Q3 2024 • Data readout: Q2 2025

As of 29 July 2025.
Appendix: Glossary.

AZD5148 (anti-TcdB mAb)

Clostridium difficile

Trial	Population	Patients	Design	Endpoints	Status
Phase I **NCT06469151**	Healthy volunteers	84	• Randomised, double-blind, placebo-controlled, dose escalation • Cohort 1: AZD5148 (dose 1, i.m.) or placebo • Cohort 2a: AZD5148 (dose 2, i.m.) or placebo • Cohort 2b: AZD5148 (dose 2, i.m., Chinese participants) or placebo • Cohort 3: AZD5148 (dose 2, i.v.) or placebo • Cohort 4a: AZD5148 (dose 3, i.v.) or placebo • Cohort 4b: AZD5148 (dose 3, i.v., Chinese participants) or placebo • Cohort 5: AZD5148 (dose 4, i.v.) or placebo	• Primary endpoint: safety • Secondary endpoint: PK parameters	• FPCD: Q2 2024 • Data anticipated: H1 2026

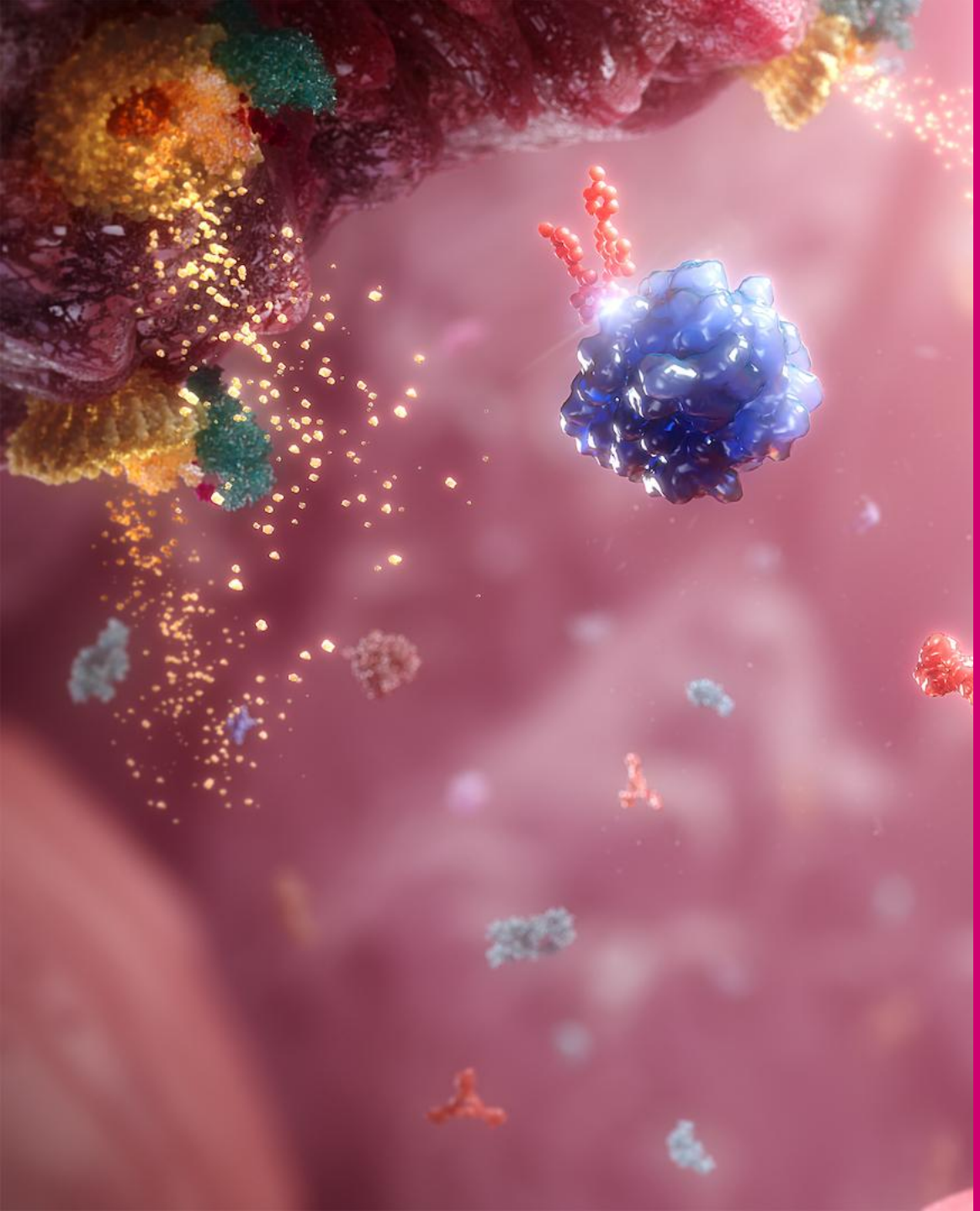
As of 29 July 2025.
Appendix: Glossary.

AZD7760 (mAb combination targeting S aureus virulence factors)

Prevention of Staph aureus infection

Trial	Population	Patients	Design	Endpoints	Status
Phase I/IIa **NCT06749457**	Phase I: healthy volunteers male and female participants aged 18 to 55 years Phase IIa: patients with ESKD receiving heamodialysis through a central venous catheter	231	• Phase I: randomised, double-blind, placebo-controlled, dose escalation trial to evaluate the safety and PK of AZD7760 to evaluate 3 doses • Phase IIa: randomised, double-blind, placebo-controlled trialto evaluate the safety and PK of AZD7760	• Primary endpoint (Phase I): safety • Primary endpoint (Phase IIa): safety • Secondary endpoints (Phase I): PK parameters and ADA • Secondary endpoints (Phase Iia): PA parameters, ADA and D451 safety	• FPCD: Q1 2025 • Data anticipated: >2026

As of 29 July 2025.
Appendix: Glossary.



Rare Disease:
approved medicines and late-stage development

AstraZeneca

Beyonttra (acoramidis, ALXN2060)

ATTR-CM

Trial	Population	Patients	Design	Endpoints	Status
Phase III **ALXN2060-TAC-302** **NCT04622046**	ATTR-CM	22	• Arm 1: 800mg *Beyonttra* administered twice daily • Japan only	• Primary endpoint: change from baseline to Month 12 of treatment in distance walked during the six-minute walk test, cause mortality and cardiovascular related hospitalisation over a 30-month period	• FPCD: Q4 2020 • Data readout: Q1 2024 • Primary endpoint met

As of 29 July 2025.
Appendix: Glossary.

Koselugo (selumetinib, MEK inhibitor)

Neurofibromatosis type 1, solid tumours

Trial	Population	Patients	Design	Endpoints	Status
Phase III **KOMET** **NCT04924608** **Partnered (Merck Sharp & Dohme LLC)**	Adult age ≥18 years with NF1 who have symptomatic, inoperable PN Available baseline chronic target PN pain score	145	• Multi-centre, international trial with a parallel, randomised, double-blind, placebo-controlled, 2 arm design • Arm 1: *Koselugo* 25mg/m2 BID • Arm 2: placebo BID until end of Cycle 12, then crossover to *Koselugo* 25mg/m2 BID	• Primary endpoint: ORR by end of Cycle 16 on *Koselugo* vs. placebo as determined by ICR per REiNS criteria • Key secondary endpoint: change from baseline of chronic PN-pain intensity on Koselugo vs. placebo	• FPCD: Q4 2021 • Data readout: Q3 2024 • Primary endpoint met
Phase I/II **SPRINKLE** **NCT05309668** **Partnered (Merck Sharp & Dohme LLC)**	Paediatric (age 1 to 7 years) diagnosed with NF1 with symptomatic, inoperable PN with at least one measurable PN, defined as a PN of at least 3cm, measured in one dimension	38	• Single-arm, open-label with *Koselugo* granule formulation	• Primary endpoints: *Koselugo* AUC0-12 derived after single dose administration [time frame: pre-dose and 1, 2, 3, 4, 6, 8 and 10-12 hours after *Koselugo* single dose on the first day of treatment (Cycle 1 Day 1)]; AEs graded by CTCAE Ver 5.0 [time frame: from screening until 30 days after last dose]	• FPCD: Q1 2022 • LPCD: Q1 2024 • Data readout: Q2 2024 • Primary endpoint met
Phase I **China PK/Safety/Efficacy** **NCT04590235** **Partnered (Merck Sharp & Dohme LLC)**	Pediatric (age 2 to 17 years old), adult NF1	32	• Single-arm trial with 3 phases: dose confirmation phase (n=6 for 3 cycles), expansion phase (24 months post-LSD) and long-term follow-up (60 months post-LSD)	• Primary endpoints: safety, tolerability and PK parameters • Secondary endpoint: efficacy (ORR, DoR; TTR; PFS)	• FPCD: Q1 2021 • Data readout: Q4 2023 • Primary endpoints met
Phase I **Food Effect/GI Tolerability** **NCT05101148** **Partnered (Merck Sharp & Dohme LLC)**	Adolescents aged ≥12 to <18 years at trial entry with a clinical diagnosis of NF1-related PN *Koselugo* with a low-fat meal compared to fasted state	24	• Single-arm, multiple dose, sequential, two or three period trial • *Koselugo* 25mg/m2 BID given with a low-fat meal vs. the same dose given in a fasted state	• Primary endpoints: PK parameters (steady state systemic exposure), safety (GI toxicity)	• FPCD: Q3 2021 • Data readout: Q4 2023 • Primary endpoints met

As of 29 July 2025.
Appendix: Glossary.

Ultomiris (anti-C5 mAb)

Haematology, nephrology, transplant

Trial	Population	Patients	Design	Endpoints	Status
Phase III **TMA-313** **NCT04543591**	Thrombotic microangiopathy-associated haematopoietic stem cell transplant	146	• Arm 1: *Ultomiris* Q8W • Arm 2: placebo	• Primary endpoint: event free survival • Secondary endpoints: overall survival, non-relapse mortality, number of TMA response criteria met	• FPCD: Q4 2020 • LPCD: Q1 2025 • Data anticipated: H2 2025
Phase III **TMA-314** **NCT04557735**	Paedeatric thrombotic microangiopathy-associated haematopoietic stem cell transplant	41	• Arm 1: *Ultomiris* administered once every 4 to 8 weeks	• Primary endpoint: proportion of participants with TMA response • Secondary endpoints: time to TMA response, proportion of participants with TMA relapse	• FPCD: Q4 2020 • LPCD: Q2 2024 • Data readout: Q1 2025 • Positive high-level results
Phase III **ARTEMIS** **NCT05746559**	CSA-AKI	736	• Randomised, double-blind, placebo-controlled, muticentre trial • *Ultomiris* i.v. to protect patients with CKD from CSA-AKI and subsequent MAKE	• Primary endpoint: to assess the efficacy of a single dose of *Ultomiris* i.v. vs. placebo in reducing the risk of the clinical consequences of AKI (MAKE) at 90 days in adult participants with CKD who undergo non-emergent cardiac surgery with CPB	• FPCD: Q1 2023 • Data anticipated: H2 2026
Phase III **I CAN** **NCT06291376**	Immunoglobulin A nephropathy	510	• Arm 1: *Ultomiris* via weight-based i.v. infusion • Arm 2: placebo via weight-based i.v. infusion	• Primary endpoints: change from baseline in proteinuria based on 24-hour UPCR at Week 34 and eGFR over 106 weeks • Secondary endpoints:, reduction in UPCR≥50%, change in proteinuria at week 1,time to sustained ≥30% eGFR decline, composite kidney endpoint	• FPCD: Q2 2024 • Data anticipated: H1 2026
Phase III **AWAKE** **NCT06830798**	Delayed graft function in high-risk donor kidneys	450	• Arm1: Placebo • Arm2: *Ultomiris*	• Primary: time to freedom from dialysis • Secondary: DGF incidence, number of dialysis sessions, time to first occurrence of eGFR => 30 mL/min/1.73m.	• FPCD: Q2 2025 • Data anticipated: >2026
Phase II **SANCTUARY** **NCT04564339**	Proliferative lupus nephritis or immunoglobulin A nephropathy	120	• Arm 1: LN cohort, *Ultomiris* • Arm 2: LN cohort, placebo • Arm 3: IgAN cohort, *Ultomiris* • Arm 4: IgAN cohort, placebo	• Primary endpoint: percentage change in proteinuria from baseline to week 26 • Secondary endpoints: percentage change in proteinuria from baseline to Week 50	• FPCD: Q1 2021 • LPCD: Q2 2025 • Data readout: Q2 2025 • Primary endpoint met (IgAN cohort) • Primary endpoint not met (LN cohort)

As of 29 July 2025.
Appendix: Glossary.

Ultomiris (anti-C5 mAb)

Neurology

Trial	Population	Patients	Design	Endpoints	Status
Phase III **NMO-307** **NCT04201262**	Neuromyelitis optica spectrum disorder	58	• Arm 1: *Ultomiris* Q8W	• Primary endpoint: time to first adjudicated on-trial relapse	• FPCD: Q4 2019 • LPCD: Q1 2021 • Data readout: Q2 2022 • Primary endpoint met
Phase II/III **NMO-317** **NCT05346354**	Neuromyelitis optica spectrum disorder	12	• Arm 1: *Ultomiris* Q8W	• Primary endpoint: change from baseline in annualised relapse rate at Week 50	• FPCD: Q3 2022 • LPCD: Q1 2025 • Data anticipated: H1 2026

As of 29 July 2025.
Appendix: Glossary.

anselamimab (CAEL-101, fibril-reactive mAb)

AL amyloidosis

Trial	Population	Patients	Design	Endpoints	Status
Phase III **CARES-302** **NCT04512235**	AL amyloidosis (Mayo Stage IIIa)	267	• Arm 1: anselamimab combined with SoC for PCD • Arm 2: placebo combined with SoC for PCD	• Primary endpoint: a hierarchical combination of time to all-cause mortality and frequency of cardiovascular hospitalisation, safety (TEAEs) • Secondary endpoint: quality of life measures	• FPCD: Q4 2020 • LPCD: Q4 2023 • Data readout: Q3 2025 • Primary endpoint not met
Phase III **CARES-301** **NCT04504825**	AL amyloidosis (Mayo Stage IIIb)	124	• Arm 1: anselamimab combined with SoC for PCD • Arm 2: placebo combined with SoC for PCD	• Primary endpoint: a hierarchical combination of time to all-cause mortality and frequency of cardiovascular hospitalisation, safety (TEAEs) • Secondary endpoint: quality of life measures	• FPCD: Q1 2021 • LPCD: Q4 2023 • Data anticipated: H2 2025 • Primary endpoint not met
Phase II **CAEL101-203** **NCT04304144**	AL amyloidosis (Mayo Stage I, Stage II and Stage IIIa)	25	• Arm 1: anselamimab combined with SoC CyBorD • Arm 2: placebo combined with SoC CyBorD and daratumumab	• Primary endpoint: occurrence of DLT during the first 4 weeks of therapy • Secondary endpoint: AUC (plasma curve concentration)	• FPCD: Q1 2020 • Data readout: Q2 2024

As of 29 July 2025.
Appendix: Glossary.

efzimfotase alfa (ALXN1850, next-generation asfotase alfa)

Hypophosphatasia

Trial	Population	Patients	Design	Endpoints	Status
Phase III **HICKORY** **NCT06079281**	Hypophosphatasia	114	• Arm 1: placebo on Day 1 followed by Q2W via s.c. injection for 24 weeks • Arm 2: bodyweight-dependent doses of either 20mg, 35mg or 50mg of efzimfotase alfa Q2W via s.c. injection for 24 weeks	• Primary endpoint: change from baseline in 6MWT at Day 169	• FPCD: Q2 2024 • LPCD: Q1 2025 • Data anticipated: H2 2025
Phase III **CHESTNUT** **NCT06079372**	Hypophosphatasia	40	• Arm 1: bodyweight-dependent doses of either 20mg, 35mg or 50mg of efzimfotase alfa Q2W via s.c. for 24 weeks • Arm 2: 6mg/kg/week of Strensiq via s.c. injection as either 2mg/kg 3 times per week or 1mg/kg 6 times per week for 24 weeks	• Primary endpoint: incidence of TEAEs	• FPCD: Q2 2024 • LPCD: Q1 2025 • Data anticipated: H2 2025
Phase III **MULBERRY** **NCT06079359**	Hypophosphatasia	30	• Arm 1: bodyweight-dependent doses of either 25mg, 35mg, or 50mg of efzimfotase Q2W via s.c. injection for 24 weeks • Arm 2: placebo Q2W for 24 weeks	• Primary endpoint: Radiographic Global Impression of Change (RGI-C) Score at Day 169	• FPCD: Q3 2024 • Data anticipated: H1 2026
Phase I **ALXN1850-HPP-101** **NCT04980248**	Hypophosphatasia	15	• Arm 1: ALXN1850, 3 cohorts at low, medium and high dosages	• Primary endpoint: incidence of TEAEs and TESAEs	• FPCD: Q3 2021 • LPCD: Q2 2022 • Data readout: Q4 2022 • Primary endpoint met

As of 29 July 2025.
Appendix: Glossary.

eneboparatide (parathyroid hormone receptor 1 agonist)

Hypoparathyroidism

Trial	Population	Patients	Design	Endpoints	Status
Phase III **CALYPSO** **NCT05778071**	Chronic hypoparathyroidism	165	• Arm 1: 20mcg eneboparatide administered once daily via s.c. injection • Arm 2: placebo administered once daily via s.c. injection	• Primary endpoint: complete independence from active vitamin D, independence from therapeutic doses of oral calcium (i.e. taking oral elemental calcium supplements ≤600mg/day) and albumin-adjusted serum calcium within the normal range (8.3 to 10.6mg/dL) vs. placebo after 24 weeks of treatment	• FPCD: Q3 2023 • Data readout: Q1 2025 • Primary endpoint met

As of 29 July 2025.
Appendix: Glossary.

gefurulimab (ALXN1720, anti-C5 dual-binding nanobody)

Neurology, nephrology

Trial	Population	Patients	Design	Endpoints	Status
Phase III **PREVAIL** **NCT05556096**	Generalised myasthenia gravis	254	• Arm 1: weight-based maintenance treatment with gefurulimab on Day 1, followed by weight-based maintenance treatment of gefurulimab on Week 1 (Day 8) and Q1W thereafter for a total of 26 weeks • Arm 2: placebo	• Primary endpoint: change from baseline in MG-ADL total score at Week 26 • Key Secondary endpoints: Change from baseline in QMG total score, Change from baseline in the MGC total score	• FPCD: Q4 2022 • LPCD: Q4 2024 • Data readout: Q3 2025 • Primary endpoint met
Phase I **ALXN1720-NEPH-102** **NCT05314231**	Proteinuria	13	• Arm 1: gefurulimab s.c. infusion at a dose of 1500mg	• Primary endpoint: serum concentration of [time frame: Day 1 (0.5 hours pre-dose and post-dose) and dose on Days 2, 3, 8, 15, 29, 43 and 57]	• FPCD: Q2 2022 • LPCD: Q1 2023 • Data readout: Q3 2023

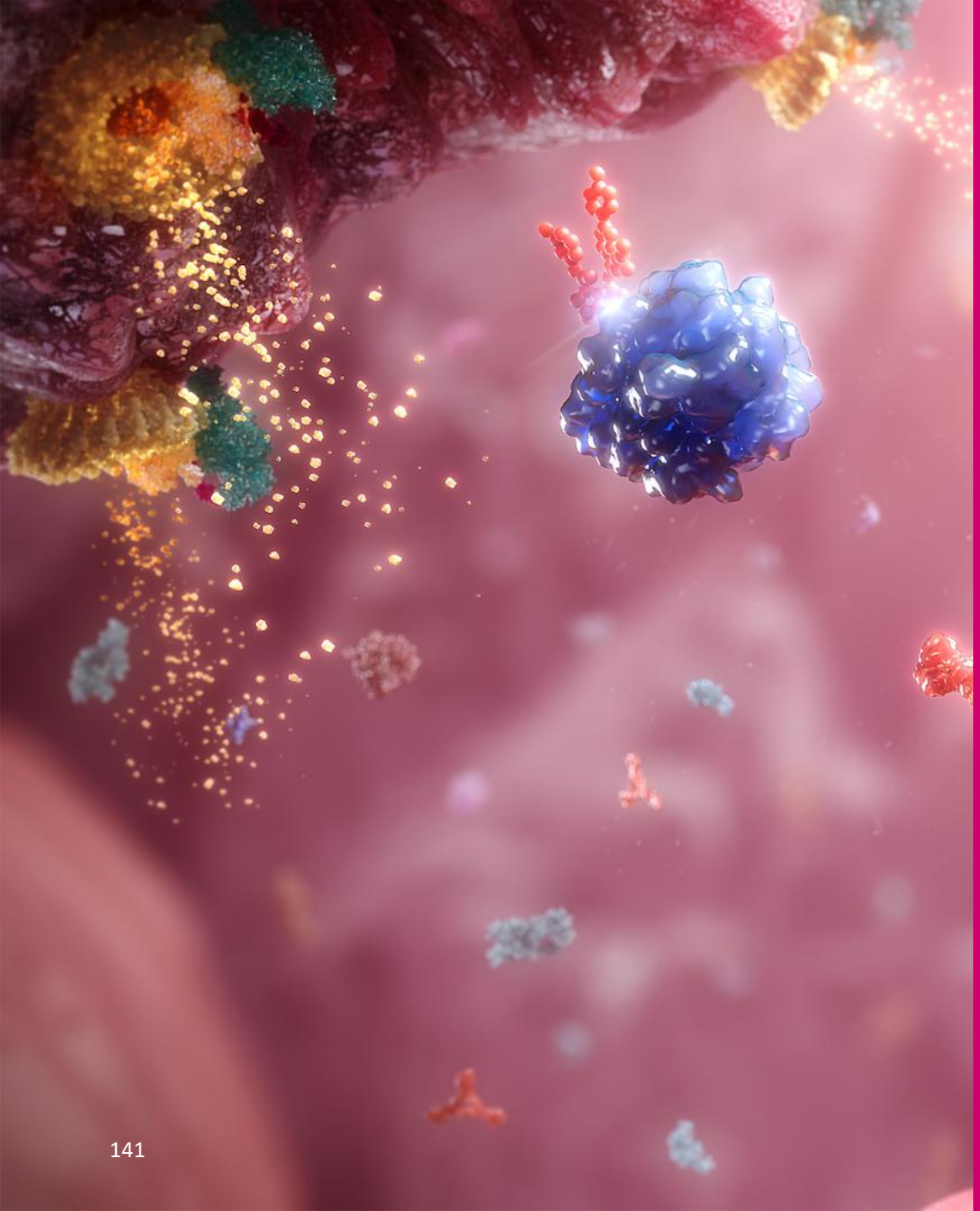
As of 29 July 2025.
Appendix: Glossary.

cliramitug (ALXN2220, TTR depleter)

Amyloidosis

Trial	Population	Patients	Design	Endpoints	Status
Phase III **DepleTTR-CM** **NCT06183931**	ATTR-CM (wild-type and variant)	1180	• Arm 1: cliramitug via i.v. infusion Q4W for at least 24 months up to a maximum of 48 months • Arm 2: placebo via i.v. infusion Q4W for at least 24 months up to a maximum of 48 months	• Primary endpoint: composite all-cause mortality and total CV events. • Secondary endpoints: KCCQ, 6MWT, all-cause mortality, CV mortality, CV events •	• FPCD: Q1 2024 • LPCD: Q2 2025 • Data anticipated: >2026

As of 29 July 2025.
Appendix: Glossary.



Rare Disease:
early-stage development

AstraZeneca

ALXN1920 (kidney-targeted factor H fusion protein)

Nephrology

Trial	Population	Patients	Design	Endpoints	Status
Phase I **ALXN1920-HV-101** **NCT05751642**	Healthy adults	48	• Randomised, double-blind, placebo-controlled SAD trial	• Primary endpoints: safety and tolerability • Secondary endpoints: PK/PD parameters	• FPCD: Q2 2023 • LPCD: Q4 2023 • Data readout: Q2 2024

As of 29 July 2025.
Appendix: Glossary.

ALXN2030 (siRNA targeting complement C3)

Transplant

Trial	Population	Patients	Design	Endpoints	Status
Phase I **ALXN2030-HV-101** **NCT05501717**	Healthy volunteers	48	• Randomised, placebo-controlled SAD trial	• Primary endpoint: safety	• FPCD: Q4 2022 • LPCD: Q2 2025 • Data anticipated: H2 2026

As of 29 July 2025.
Appendix: Glossary.

MEDI1341 (alpha-synuclein mAb)

Multiple system atrophy

Trial	Population	Patients	Design	Endpoints	Status
Phase II **NCT05526391** **Partnered (Takeda)**	Patients with diagnosis of possible or probably MSA (using modified Gilman et al. 2008 diagnostic criteria)	158	• Randomised, double-blind, placebo-controlled trial • Early PK cohort • Arm 1: MEDI1341 i.v. • Arm 2: placebo i.v. • Main cohort • Arm 3: MEDI1341 i.v. • Arm 4: placebo i.v.	• Primary endpoints: efficacy, change from baseline on modified Unified Multiple System Atrophy Rating Scale at 52 weeks • Secondary endpoints: PK parameters, safety and efficacy	• FPCD: Q4 2022 • LPCD: Q2 2024 • Data anticipated: H2 2025

Glossary – 1 of 5

14C	Carbon 14
1L, 2L, 3L	1st-, 2nd- or 3rd-line
5-FU	5-fluorouracil
6MWT	6-minute walk test
A2AR	Adenosine A2A receptor
AAV	Adeno-associated virus
ACE	Angiotensin-converting enzyme
AChR+	Acetylcholine receptor-positive
ACQ	Asthma Control Questionnaire
ACR	American College of Rheumatology Response Scoring System
ADA	Anti-drug antibody
ADC	Antibody-drug conjugate
ADP	Adenosine diphosphate
ADsCa	Albumin-adjusted serum calcium
AE	Adverse event
AER	Annual exacerbation rate
AEs	Adverse effects
AGA	Actional genomic alteration
aHUS	Atypical haemolytic uraemic syndrome
AI	Auto-injector
AI	Aromatase inhibitor
AKT	Protein kinase B
AL amyloidosis	Light-chain amyloidosis
ALK	Anaplastic large-cell lymphoma kinase
ALL	Acute lymphocytic leukaemia
alloSCT	Allogeneic stem cell tranplantation
ALSFRS-R	Amyotrophic Lateral Sclerosis Functional Rating Scale-Revised
AML	Acute myeloid leukaemia
AMR	Antibody mediated rejection
anti-FRα	Anti-folate receptor alpha
anti-PCD	Anti-plasma cell dyscrasia
APFS	Accessorised pre-filled syringe
APOL1	Apolipoprotein L1
APOL1 G0/G1/G2	Sequences of the G0, G1, and G2 APOL1 variants from amino acids 339–398
AQLQ	Asthma Quality of Life Questionnaire
AQP4+	Aquaporin-4 antibody positive
ARB	Angiotensin receptor blockers
AS	Albuterol sulfate
ASCO	American Society of Clinical Oncology
ASI	Aldosterone synthase inhibitor
ASO	Antisense oligonucleotide
ATM	Ataxia telangiectasia mutated kinase
ATR	Ataxia telangiectasia and Rad3-related protein
ATTR	Transthyretin amyloidosis
ATTR-CM	Transthyretin amyloid cardiomyopathy
ATTR-PN	Transthyretin amyloid polyneuropathy
ATTRv-PN	Hereditary transthyretin-mediated amyloid polyneuropathy
AUC	Area under curve
AUCinf	Area under plasma concentration time curve from zero to infinity
AUClast	Area under plasma concentration curve from zero to the last quantifiable concentration
AUCt	Area under concentration-time curve
AUEC	Area under the effect-time curve
Avb8	Alpha v beta 8
B7H4	B7 homolog 4
BA	Bioavailability
BAFF	B-cell activating factor
B-ALL	B cell acute lymphoblastic leukaemia
BBB	Blood-brain barrier
BCG	Bacillus Calmette-Guérin
BCL2	B-cell leukemia/lymphoma 2 protein
BCMA	B-cell maturation antigen
BDA	Budesonide albuterol
BFF	Budesonide and formoterol fumarate
BGF	Budesonide, glycopyrronium and formoterol fumarate
BICLA	British Isles Lupus Assessment Group-based Composite Lupus Assessment
BICR	Blinded independent central review
BID	Twice per day
BIG	Big Ten Cancer Research Consortium
BM	Biomarker
BMD	Bone mineral density
BMFI	Bone metastasis-free interval
BMI	Body mass index
BOR	Best overall response rate
BR	Bendamustine and rituximab
BRCA	BReast CAncer gene
BRCAm	BReast CAncer gene-mutated
BRCAwt	BReast CAncer wild-type gene
BRD4	Bromodomain-containing protein 4
BTC	Biliary tract carcinoma
BTC	Biliary tract cancer
BTK	Bruton's tyrosine kinase
BTKi	Bruton's tyrosine kinase
BVAS	Birmingham Vasculitis Activity Score
C3	Complement component 3
C5	Complement component 5
CA-125	Cancer antigen-125
CAAT	Chronic Airways Assessment Test
CAD	Coronary artery disease
CAGR	Compound annual growth rate
cAMR	Chronic antibody-medicated rejection
CAR-T	Chimeric antigen receptor therapy
CBP	Cardiopulmonary bypass
CBR	Clinical benefit rate
CD	Cluster of differentiation
CD123	Interleukin 3 receptor a
CD19	Cluster of differentiation 19
CD3	Cluster of differentiation 3
CD39	Cluster of differentiation 39
CD73	Cluster of differentiation 73
CD8	Cluster of differentiation 8
CDAI	Clinical Disease Activity Index
CDK	Cyclin-dependent kinase
CDK2	Cyclin-dependent kinase 2
CDK4/6i	Cyclin-dependent kinase 4/6 inhibitor
CE	Clinically evaluable
CHD	Coronary heart disease
Chemo	Chemotherapy
CHF	Chronic heart failure
cHL	Classic Hodgkin lymphoma
CI	Confidence interval
CKD	Chronic kidney disease
CLD	Chronic lung disease
CLDN 18.2	Claudin-18.2
CLDN18.2	Claudin 18.2
CLL	Chronic lymphocytic leukaemia
cm	Centimetre
CM	Cardiomyopathy
CMAX	Maximum observed plasma concentration
cMET	C-mesenchymal epithelial transition factor
CMML	Chronic myelomonocytic leukaemia

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CNS	Central nervous system
CNS-PFS	Central nervous system progression-free survial
CompEx	Composite endpoint for exacerbations
COPD	Chronic obstructive pulmonary disease
CPB	Cardiopulmonary bypass
CPI	Checkpoint inhibitor
CPI-experienced	Checkpoint inhibitor-experienced
CPI-naive	Checkpoint inhibitor-naïve
cPR	Central pathological review
CR	Complete response
CRC	Colorectal cancer
CrCl	Creatinine clearance
CRR	Complete response rate
CRR	Complete renal response
CRSwNP	Chronic rhinosinusitis with nasal polyps
CRT	Chemoradiotherapy
CRwNP	Chronic rhinosinusitis with nasal polyps
CSA-AKI	Cardiac surgery-associated acute kidney injury
CTC	Circulating tumour cell
CTCAE	Common Terminology Criteria for Adverse Events
ctDNA	Circulating tumour DNA
CTLA4	Cytotoxic T-lymphocyte associated protein 4
CTLA-4	Cytotoxic T-lymphocyte-associated antigen-4
CTx	Chemotherapy
CV	Cardiovascular
CVOT	Cardiovascular outcomes trial
CVRM	Cardiovascular, Renal and Metabolism
CXCR2	C-X-C Motif chemokine receptor 2
CyBorD	Cyclophosphamide, bortezomib and dexamethasone
Datroway	Datroway
DCR	Disease control rate
DDFS	Distant disease-free survival
DDI	Drug-drug Interaction
DDR	DNA damage response
dECG	Differentiated electrocardiogram
DFS	Disease-free survival
DGF	Delayed graft function
DLBCL	Diffuse large B-cell lymphoma
DLT	Dose-limiting toxicity
DMARDs	Disease-modifying antirheumatic drugs
DNA	Deoxyribonucleic acid
dNCC	Directly measured non-ceruloplasmin-bound copper
dnTGFb	Dominant-negative transforming growth factor-beta
DoCR	Durability of complete response
DoR	Duration of response
DPB	Disease progression in bone
DPI	Dry powder inhaler
dPTEN	Phosphatase and tensin homolog deficient
DRFI	Disease recurrence-free interval
DSQ	Dysphagia Symptom Questionnaire
DXA	Dual energy X-ray absorptiometry
EBITDA	Earnings before interest, tax, depreciation and amortisation
EBRT	External beam radiation therapy
ECG	Electrocardiogram
ED	Emergency department
EFS	Event-free survival
EG	Eosinophilic gastritis
EGE	Eosinophilic gastroenteritis
eGFR	Estimated glomerular filtration rate
eGFR	Epidermal growth factor receptor-mutated
EGFRi	Epidermal growth factor receptor inhibitor
EGFRm	Epidermal growth factor receptor-mutated
EGPA	Eosinophilic granulomatosis with polyangiitis
EM	Emerging Markets
EoE	Eosinophilic oesophagitis
EOS	Eosinophil
EPI	Epigenetics
ER	Estrogen receptor
ER+	Estrogen receptor-positive
ERK	Extracellular signal-regulated kinase
ERoW	Established Rest of World
E-RS:COPD	Evaluating Respiratory Symptoms in Chronic Obstructive Pulmonary Disease
ERT	Enzyme replacement therapy
ESAI	Eczema Area and Severity Index
ESCC	Esophageal squamous cell carcinoma
ESKD	Early-stage kidney disease
ESR1	Estrogen receptor 1
ESRD	End-stage renal disease
ET	Endocrine therapy
ETA	Endothelin A
ETA	Endothelin A
ETA RA	Endothelin receptor A antagonist
EU	European Union
EVH	Extravascular haemolysis
FAF	Fundus autofluorescence
FCR	Fludarabine, cyclophosphamide and rituximab
FDC	Fixed-dose combination
FeNO	Fractional nitric oxide concentration in exhaled breath
FEV	Forced-expiratory volume
FEV1	Forced expiratory volume in 1 second
FGFR	Fibroblast growth factor receptor
FL	Follicular lymphoma
FLAP	5-lipoxygenase activating protein
FLOT	Fluorouracil, leucovorin, oxaliplatin and docetaxel
FOLFOX	Folinic acid, fluorouracil and oxaliplatin
FOXP3	Forkhead box P3
FP	5-fluorouracil/cisplatin
FPCD	First patient commenced dosing
FPG	Fasting plasma glucose
FRα	Folate receptor alpha
FX	Foreign exchange
G7	US, Japan, EU5
GA	Geographic atrophy
GBM	Glioblastoma
gBRCAm	Germline BRCA-mutated
GC	Gastric cancer
GCB	Germinal center B-cell
GEJ	Gastric/gastroesophogeal junction
GEJC	Gastroesophageal junction cancer
GFF	Glycopyrronium and formoterol fumarate
GI	Gastrointestinal
GLP-1	Glucagon-like peptide-1
GLP-1/glu	Glucagon-like peptide 1 receptor/glucagon dual peptide agonist
GLP-1RA	Glucagon-like peptide 1 receptor agonist
GMFR	Geometric mean fold rise
gMG	Generalised myasthenia gravis
GMT	Geometric mean titer
GN	Glomerulonephritis
GPC3	Glypican-3
GPC3-positive	Glypican 3-positive

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Appendix: Glossary.

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GPRC5D	G protein-coupled receptor, class C, group 5, member D
GU	Genitourinary
GYN	Gynaecologic
H1	H1-antihistamine
hADME	Human mass balance
HbA1c	Glycated haemoglobin
HCC	Hepatocellular carcinoma
HD	High dose
HDL-C	High-density lipoprotein cholesterol
HER2	Human epidermal growth factor receptor 2
HER2-low	Human epidermal growth factor receptor 2-low
HER2-negative	Human epidermal growth factor receptor 2-negative
HER2-positive	Human epidermal growth factor receptor 2-positive
HES	Hyper eosinophilic syndrome
HF	Heart failure
HFA	Hydrofluoroalkane
HFpEF	Heart failure with preserved ejection fraction
HFrEF	Heart failure with reduced ejection fraction
HGFR	Met/hepatocyte growth factor receptor
HGSC	High-grade serous carcinoma
hHF	Hospitalisation for heart failure
HIF-PH	Hypoxia inducible factor-prolyl hydroxylase
HK	Hyperkalaemia
HLA-A*02:01	Human leukocyte antigen serotype within the HLA-A serotype group
HLR	High-level results
hMPV	Human metapneumovirus
HNSCC	Head and neck squamous-cell carcinoma
HPD	Hyperprogressive disease
HPDD	Highest protocol-defined dose
HPF	High-power field
HPP	Hypophosphatasia
HR	Hazard ratio
HR+	Hormone receptor-positive
HRD	Homologous recombination deficiency
HRD+	Homologous recombination deficiency-positive
HR-low	Hormone receptor-low
HRR	homologous recombination repair
HRRm	Homologous recombination repair-mutated
HSCT-TMA	hematopoietic stem cell transplantation-associated thrombotic microangiopathy
HSD17B13	Hydroxysteroid 17-beta dehydrogenase 13
HVPG	Hepatic venous pressure gradient
i	Inhibitor
i.m.	Intramuscular
i.v.	Intravenous
IA	Investigator-assessed
IBD	Inflammatory bowel disease
ICR	Independent central review
ICS	Inhaled corticosteroid
ICS-LABA	Inhaled corticosteroid long-acting beta-agonists
ICU	Intensive care unit
IDFS	Invasive disease-free survival
IgAN	Immunoglobulin A nephropathy
IHF	Impaired hepatic function
IIT	Investigated initiated trial
iJAK1	Inhaled Janus kinase
IL	Interleukin
IL-12	Interleukin-12
IL-33	Interleukin-33
IL-5	Interleukin-5
IL-5R	Interleukin-5 receptor
IMAC-TIS	International Myositis Assessment And Clinical Studies-Total Improvement Score
IND	Investigational new drug
INV	Investigator review
IO	Immuno-oncology
IPF	Idiopathic pulmonary fibrosis
IPFS	Invasive progression-free survival
IRA	Inflation Reduction Act
IRAK4	Interleukin-1 receptor-associated kinase 4
IRC	Independent review committee
ISS	Investigator-sponsored studies
ISS7	Itch-severity score (weekly)
iTSLP	Inhaled thymic stromal lymphopoietin
ITT	Intent-to-treat
IVIg	Intravenous immunoglobulin
JAK-1	Janus kinase 1
K+	Potassium
KCCQ	Kansas City Cardiomyopathy Questionnaire
kg	Kilogram
Ki67	Antigen Kiel 67
LA amylin	Long-acting amylin
LAAB	Long-acting antibody
LABA	Long-acting beta agonist
LAMA	Long-acting muscarinic agonist
LCAT	Lecithin-cholesterol acyltransferase
LCM	Lifecycle management
LDH	Lactate dehydrogenase
LDL-C	Low-density lipoprotein cholesterol
LICA	Ligand-conjugated ASO
LIF	Low-density lipoprotein cholesterol
LN	Lupus nephritis
LoE	Loss of exclusivity
LOS	Length of stay
LPCD	Last patient commenced dosing
LSD	Last subject dosed
LS-SCLC	Limited stage small-cell lung cancer
LV	Left ventricle
m	Mutation
mAb	Monoclonal antibody
MABA	Muscarinic antagonist-beta2 agonist
MACE	Major adverse cardiac events
MAD	Multiple ascending dose
MAKE	Major adverse kidney events
MASH	Metabolic dysfunction-associated steatohepatitis
MASLD	Metabolic dysfunction-associated steatotic liver disease
mBC	Metastatic breast cancer
MCC	Mucociliary clearance
MCL	Mantle cell lymphona
mCRPC	Metastatic castrate-resistant prostate cancer
MDI	Metered-dose inhaler
mDOR	Median duration of response
MDS	Myelodysplastic syndrome
MEK	Mitogen-activated protein kinase
MET	Mesenchymal epithelial transition factor
mFOLFOX	Modified folinic acid, fluorouracil and oxaliplatin
mg	Milligram
mg/dL	Milligrams per decilitre
MG-ADL	Myasthenia Gravis-Activities of Daily Living
MGFA	Myasthenia Gravis Foundation of America
mHSPC	Metastatic hormone sensitive prostate cancer

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Appendix: Glossary.

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MI	Myocardial infarction
mL	Millilitre
MM	Multiple myeloma
MMAE	Monomethyl auristatin E
MMT	Mixed meal test
MoA	Mechanism of action
mPFS	Median progression-free survival
MPO	Myeloperoxidase
mPR	Major pathological response
MR	Mineralocorticoid receptor
MRA	Mineralocorticoid receptor antagonist
MRD-negative	Minimal residual disease-negative
MRI	Magnetic resonance imaging
MRM	Mineralocorticoid receptor modulator
mRNA	Messenger ribonucleic acid
MSA	Multiple system atrophy
MTAP-deficient	Methylthioadenosine phosphorylase-deficient
MTD	Maximum tolerated dose
mTNBC	Metastatic triple-negative breast cancer
MZL	Marginal zone lymphoma
n/m	Not material
nAb	Neutralising antibody
NaC	Sodium channel
NAFLD	Non-alcoholic fatty liver disease
NASH	Non-alcoholic fatty liver disease
NBRx	New-to-brand prescription
NCFB	Non-cystic fibrosis bronchiectasis
NCI	National Cancer Institute
NCPV	Noncalcified plaque volume
Neo-adj	Neoadjuvant
NF1	Neurofibromatosis type 1
NF1-PN	Neurofibromatosis type 1 with plexiform neurofibromas
ng	Next-generation
NGF	Nerve growth factor
ngSERD	Next-generation oral selective estrogen receptor degrader
NHA	Novel hormonal agent
NHL	Non-Hodgkin's lymphoma
NIH	National Institute of Health
NKTCL	Extranodal natural killer T-cell lymphoma
NME	New molecular entity
NME	New molecular entity
NMOSD	Neuromyelitis optica spectrum disorder
NP	Nasal polyps
NRDL	National Reimbursement Drug List
NRG	National Clinical Trials Network in Oncology
NSCLC	Non-small cell lung cancer
NST	Neoadjuvant systemic treatment
NT-proBNP	N-terminal pro-B-type natriuretic peptide
NYHA	New York Heart Association
OBD	Optimal biological dose
OCS	Oral corticosteroid
OD	Once daily
oGLP1	Oral glucagon-like receptor peptide 1
OGTT	Oral glucose tolerance test
oPCSK9	Oral protein convertase subtilisin/kexin type 9
OR	Objective response
ORR	Overall response rate
oRXFP1	Oral relaxin family peptide receptor 1
OS	Overall survival
PA	Primary aldosteronism
PALB2m	Partner and localizer of BRCA2-mutated
PAR2	Protease-activated receptor 2
PARP	Poly ADP ribose polymerase
PARP1	poly(ADP-ribose) polymerase-1
PARP-1sel	Poly ADP ribose polymerase-1 selective
PARPi	poly-ADP ribose polymerase inhibitor
PASI	Psoriasis area severity index
PBD	Pyrrolobenzodiazepine
PCD	Plasma cell dyscrasia
pCR	Pathological complete response
PCSK9	Proprotein convertase subtilisin/kexin type 9
PD	Pharmacodynamics
PD1	Programmed cell death protein 1
PD-1	Programmed cell death protein-1
PDAC	Pancreatic ductal adenocarcinoma
PDE4	Phosphodiesterase type 4
PD-L1	Programmed death-ligand 1
PD-L1-high	Programmed death-ligand 1-high
Peak	Maximum
PET	Positron-emission tomography
PFS	Progression-free survival
PFS2	Time to second disease progression or death
PgR	Progesterone receptor
PI3K	Phosphoinositide 3 kinase
PIK3CA	Phosphatidylinositol-4,5-biphosphate 3-kinase catalytic subunit
PK	Pharmacokinetic
PK/PD	Pharmacokinetic/pharmacodynamic
PLEX	Plasma exchange
PLL	Prolymphocytic leukaemia
pMDI	Pressurised metered-dose inhaler
PN	Plexiform neurofibroma
PN	Polyneuropathy
PNH	Paroxysmal nocturnal haemoglobinuria
PNH-EVH	PNH with extravascular haemolysis
PNPLA3	Phospholipase domain-containing protein 3
POC	Proof-of-concept
POM	Proof-of-mechanism
post-BD	Post-bronchodilator
PP	Plasmapheresis
pPCI	Primary percutaneous coronary intervention
PR	Partial response
pre-BD	Pre-bronchodilator
PRMT5	Protein arginine methyltransferase 5
PRO	Patient reported outcome
PRR	Recurrent platinum resistant
PS	Propensity score
PSA	Prostate-specific antigen
PSA50	Prostate-specific antigen 50
PSC	Pulmonary sarcomatoid carcinoma
PSMA	Prostate-specific membrane antigen
PSR	Platinum-sensitive relapsed
PTCL	Peripheral T-cell lymphoma
PTEN	Phosphatase and tensin homolog gene
PTH	parathyroid hormone receptor
PVR	Pulmonary vascular resistance
Q1W	Every one week
Q2W	Every two weeks
Q4W	Every four weeks
Q8W	Every eight weeks
QCS	Quantitative continuous scoring

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Appendix: Glossary.

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QD	Once daily
QID	Four times per day
QOD	Every other day
QoL	Quality of life
QoL-DN	Norfolk Quality of Life-Diabetic Neuropathy
QT	Duration of ventricular electrical systole
QTcF	Corrected QT interval by Fredericia
R&I	Respiratory and Immunology
R/R	Relapsed/refractory
r/r	Relapsed/refractory
RA	Rheumatoid arthritis
RAAS	Renin-angiotensin-aldosterone system
RAGE	Receptor for advanced glycation end products
RC	Radioconjugates
RECIST	Response Evaluation Criteria in Solid Tumours
REiNS	Response Evaluation in Neurofibromatosis and Schwannomatosis
RET	Rearranged during transfection
RFS	Relapse-free survival
rhLCAT	Recombinant human lecithin-cholesterol acyltransferase
rNDV	Recombinant Newcastle disease virus
RORγ	Related orphan receptor gamma
RP2D	Recommended Phase II dose
rPFS	Radiographic progression-free survival
RR	Response rate
RSV	Respiratory syncytial virus
RT	Radiation therapy
s. asthma	Severe asthma
s.c.	Subcutaneous
SABA	Short-acting beta2-agonist
SAD	Single ascending dose
SAE	Serious adverse event
SARS-CoV-2	Severe-acute-respiratory-syndrome-related coronavirus-19
SBP	Systolic blood pressure
SBRT	Stereotactic body radiation therapy
SCCHN	Squamous-cell carcinoma of the head and neck
SCD	Sickle cell disease
SCLC	Small cell lung cancer
SD	Stable disease
SERD	Selective estrogen receptor degrader
SG&A	Selling, General and Administrative
SGLT2	Sodium-glucose transport protein 2
SGLT2i	Sodium/glucose cotransporter 2 inhibitor
SGRM	Selective glucocorticoid receptor modulator
SGRQ	Saint George Respiratory Questionnaire
siRNA	Small interfering ribonucleic acid
SJC	Swollen joint count
sK	Serum potassium
SLE	Systemic lupus erythematosus
SLL	Small lymphocytic lymphoma
SMAD	Single and multiple ascending dose trial
SoC	Standard-of-care
sPGA	Static Physician's Global Assessment Score
SS	Steady state
ST2	Suppression of tumourigenicity 2
STAT3	Signal transducer and activator of transcription 3
Stg. I/II/III	Stage I/II/III
sUA	Serum uric acid
T2D	Type-2 diabetes
T2DM	Type-2 diabetes mellitus
T300	Imfinzi plus Imjudo
T790M	Threonine 790 substitution with methionine
TACE	Transarterial chemoembolization
tBRCAm	Tumour (somatic) BRCA-mutated
TCE	T-cell engager
TCR	T-cell receptor
TCR-T	T-cell receptor therapy
TDR	Tumour drivers and resistance
TEAE	Treatment-emergent adverse event
TESAE	Treatment-emergent serious adverse event
TFST	Time to first subsequent therapy or death
TGFbetaRIIDN	Transforming growth factor-beta RIIDN
THP	Paclitaxel, trastuzumab and pertuzumab
TID	Three times per day
TIGIT	T-cell immunoreceptor with Ig and ITIM domains
TIM3	T-cell immunoglobulin and mucin domain 3
TIM-3	T-cell immunoglobulin and mucin domain-containing protein
TJC	Tender joint count
TKI	Tyrosine kinase Inhibitor
TLR	Toll-like receptor 9
TMA	Thrombotic microangiopathy
Tmax	Time to reach maximum observed plasma concentration
TNBC	Triple negative breast cancer
TNF	Tumour necrosis factor
TNSALP	Tissue-nonspecific alkaline phosphatase
TOP1i	Topoisomerase 1 inhibitor
TP53	Tumour protein 53
TP53 R175H	Tumour protein p53 with arginine at position 175 is replaced with histidine
TPS	Tumour proportion score
Treg	Regulatory T-cell
TROP2	Trophoblast cell surface antigen 2
TSLP	Thymic stromal lymphopoietin
TTD	Time to treatment discontinuation
TTF	Time to treatment failure
TTNT	Time to next therapy
TTP	Time to tumour progression
TTR	Time to treatment response
TTR	Transthyretin
u/r HTN	Uncontrolled or treatment resistant hypertension
UACR	Urinary albumin/creatinine ratio
UK	United Kingdom
ULN	Upper limit of normal
u-LTE4	Urinary leukotriene E4
UMEC	Umeclidinium
UPCR	Urine protein creatinine ratio
URAT1	Uric acid transporter 1
US	United States
V&I	Vaccines and Immune Therapies
VEGF	Vascular endothelial growth factor
VHH	Single domain antibody
VLP	Virus-like particle
XELOX	Oxaliplatin and capecitabine

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Appendix: Glossary.