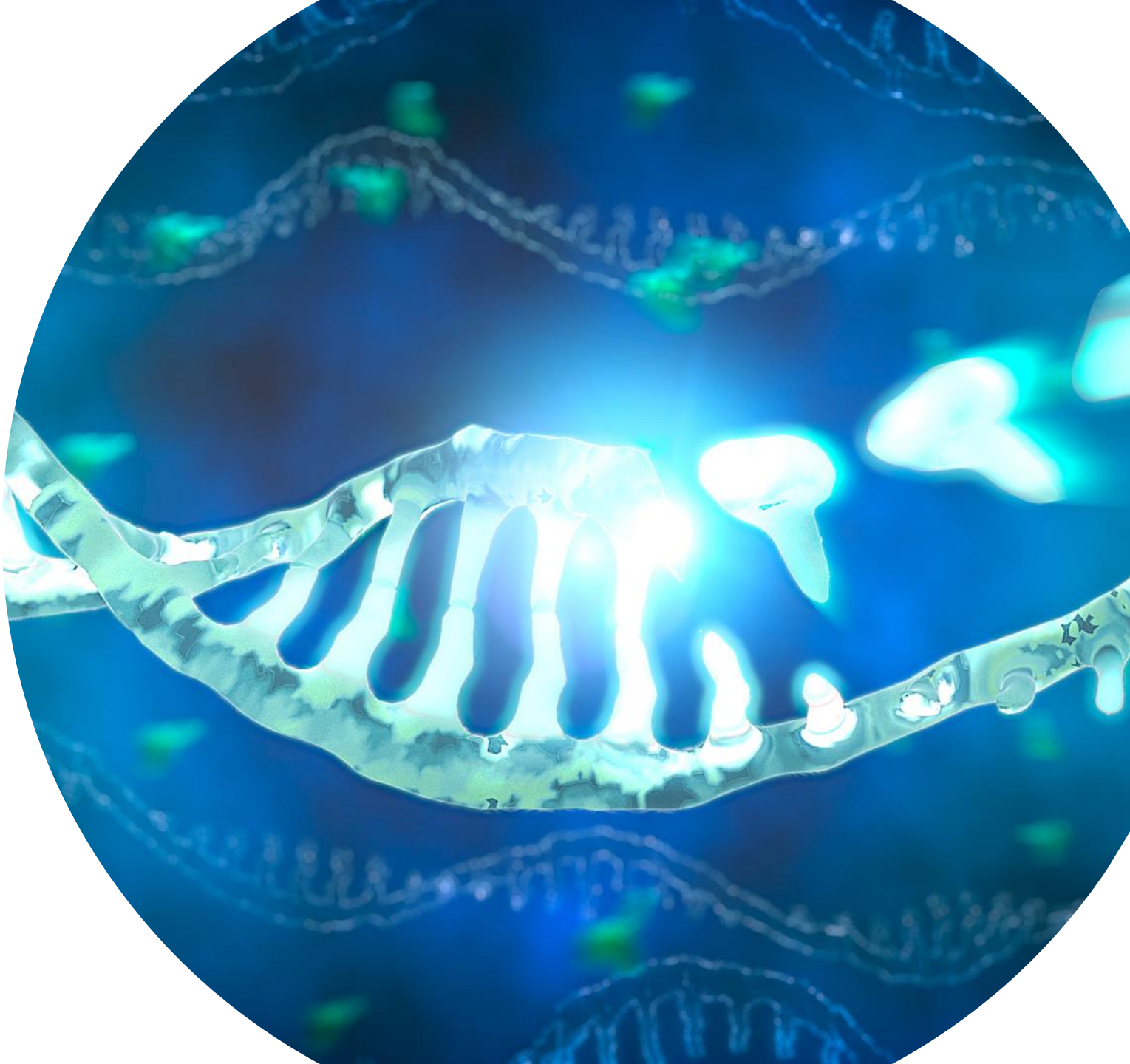
AstraZeneca

Clinical Trials Appendix

Q1 2024 Results Update

25 April 2024



Pipeline
at a glance

Across five focus therapy areas:

- **Oncology**
- **BioPharmaceuticals**
  CVRM | R&I | V&I
- **Rare Disease**

182

projects in our development pipeline

19

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

123

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

9

regulatory approvals in major markets since FY 2023

As of 25 April 2024.
Appendix: Glossary.

Key upcoming pipeline catalysts: 2024 and 2025

Oncology **BioPharmaceuticals** **Rare Disease**

	H1 2024	H2 2024	2025	
Regulatory decision[1,2]	***Imfinzi*** – NSCLC (neoadjuvant) (AEGEAN) ***Truqap*** – HR+/HER2-neg breast cancer (2L) (CAPItello-291) (EU) **roxadustat** – chemotherapy-induced anaemia (CN)	***Tagrisso*** – EGFRm NSCLC (1L) (FLAURA2) (EU, CN) ***Lynparza + Imfinzi*** – endometrial cancer (1L) (DUO-E) ***Enhertu*** –HER2+/HER2-low gastric cancer (3L) (DESTINY-Gastric01) (CN) **Dato-DXd** – NSCLC (2L and 3L) (TROPION-Lung01) ***Wainua*** – ATTRv-PN (NEURO-TTRansform) (EU) ***Fasenra*** – EGPA (MANDARA) ***Fasenra*** – asthma (MIRACLE) (CN)	***Lynparza*** – gBRCA breast cancer (adjuvant) (OlympiA) (CN) ***Truqap*** – HR+/HER2-neg breast cancer (2L) (CAPItello-291) (CN) **Dato-DXd** – HR+/HER2- breast cancer (inoperable and/or met.) (TROPION-Breast01) ***Ultomiris*** – gMG (CN) **acoramidis** – ATTR-CM (ALXN2060-TAC-302) (JP)	
Key Phase III data readouts	***Enhertu*** – HER2-low breast cancer (2L) (DESTINY-Breast06) ***Truqap*** – TNBC (locally adv./met.) (CAPItello-290) ***Fasenra*** – HES (NATRON) **sipavibart** – prevention of COVID-19 (SUPERNOVA)	***Tagrisso*** – EGFRm NSCLC (resectable, Stg. II/III) (NeoADAURA) ***Imfinzi*** – NSCLC (unresectable) (Stg. III) (PACIFIC-5) ***Imfinzi*** – bladder cancer (1L) (NILE) ***Lynparza*** – PARP BRCAwt ovarian cancer (1L) (MONO-OLA1) ***Orpathys*** – NSCLC with MET exon 14 mutations (locally adv./met.) **Dato-DXd** – TNBC (locally rec. inop./met.) (TROPION-Breast02) ***Truqap*** – de novo PTEN deficient met. HSPC (CAPItello-281) ***Fasenra*** – CRwNP (ORCHID) ***Tezspire*** – CRwNP (WAYPOINT) ***Tezspire*** – severe asthma (DIRECTION) ***Koselugo*** – NF1-PN (KOMET)	***Tagrisso*** – EGFRm NSCLC (SAFFRON) ***Imfinzi*** – non-muscle-inv. bladder cancer (POTOMAC) ***Imfinzi*** – bladder cancer (muscle invasive) (NIAGARA) ***Imfinzi*** – GC/GEJC (resect.) (MATTERHORN) ***Imfinzi*** – muscle-inv. bladder cancer (VOLGA) ***Enhertu*** – high-risk HER2+ early breast cancer (DESTINY-Breast05) ***Enhertu*** – HER2+ met. breast cancer (1L) (DESTINY-Breast09) ***Enhertu*** – high-risk HER2+ early breast cancer (non-met.) (DESTINY-Breast11) ***Enhertu*** – HER2+ gastric cancer (2L) (DESTINY-Gastric04) ***Enhertu*** – HER2m NSCLC (DESTINY-Lung04) ***Calquence*** – previously untreated CLL (AMPLIFY) ***Calquence*** – previously untreated MCL (ECHO) **camizestrant** – HR+/HER2-neg breast cancer (SERENA-6) **ceralasertib** – post-IO NSCLC (LATIFY) **Dato-DXd** – NSCLC (1L) (AVANZAR)	**baxdrostat** – uncontrolled hypertension (BaxHTN) ***Breztri*** – mild to moderate asthma (LITHOS) ***Breztri*** – severe asthma (KALOS) ***Breztri*** – severe asthma (LOGOS) ***Breztri*** – moderate asthma (VATHOS) ***Breztri*** – COPD (ATHLOS) ***Fasenra*** – moderate to severe COPD (RESOLUTE) ***Saphnelo*** – moderate to severe SLE (TULIP-SC) ***Saphnelo*** – moderate to severe SLE (AZALEA-SLE) ***Airsupra*** – mild asthma (BATURA) ***Ultomiris*** – HSCT-TMA (ALXN1210-TM-313) **anselamimab** – AL amyloidosis (Mayo Stg. IIIa) (CAEL101-302) **anselamimab** – AL amyloidosis (Mayo Stg. IIIb) (CAEL101-301)

[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 25 April 2024.
Appendix: Glossary.

Clinical Trials Appendix: selected highlights

	BioPharmaceuticals	Oncology	Rare Disease
Approved medicines: key LCM	farxiga (dapagliflozin) Fasenra (benralizumab) AIRSUPRA (albuterol 90 mcg/budesonide 80 mcg) Inhalation Aerosol BREZTRI AEROSPHERE Saphnelo (anifrolumab-fnia) WAINUA (eplontersen) TEZSPIRE (tezepelumab-ekko)	TAGRISSO osimertinib ENHERTU fam-trastuzumab deruxtecan-nxki CALQUENCE (acalabrutinib) 100 mg capsules Lynparza olaparib IMFINZI durvalumab Truqap capivasertib 160 mg • 200 mg tablets	ULTOMIRIS (ravulizumab-cwvz)
Next-wave pipeline	tozorakimab (IL-33 ligand mAb) sipavibart (AZD3152, COVID-19 LAAB) mitiperstat (MPO inhibitor) baxdrostat (aldosterone synthase inhibitor)	Dato-DXd (TROP2 ADC) camizestrant (oral SERD) saruparib (PARP1 inhibitor) *bispecific mAbs:* volrustomig (PD-1/CTLA-4); rilvegostomig (PD-1/TIGIT); sabestomig (PD-1/TIM3)	vemircopan (factor D inhibitor) gefurulimab (humanised bispecific heavy-chain antibody) efzimfotase alfa (ALXN1850, ngHPP) ALXN2220 (TTR depleter)

As of 25 April 2024.
Appendix: Glossary.

Project movements since Q4 2023 update

New to Phase I

NME

AZD0292 (pseudomonas PsI-PcrV bispecific mAb) non-CF bronchiectasis

AZD0754[#] (STEAP2 CAR-T) prostate cancer

AZD1705
lipid lowering cardiovascular disease

AZD0120 (autologous anti-CD19 and anti-BCMA CART cell immunotherapy) multiple myeloma

NT-112[#] (TGFBR2 KO armored TCR-T targeting KRAS G12D) solid tumours

New to Phase II

New to pivotal trial

NME

AZD0901 (CLDN18.2 MMAE ADC)
CLARITY-Gastric01 gastric cancer (2L+)

balcinrenone/dapagliflozin (MR modulator and SGLT2 inhibitor) heart failure with CKD

Additional indication

baxdrostat/dapagliflozin (aldosterone synthase inhibitor and SGLT2 inhibitor) CKD

datopotamab deruxtecan (TROP2 ADC)
TROPION-Lung10[#] locally advanced or metastatic non-squamous NSCLC with high PD-L1 expression and without actionable genomic alterations

Life-cycle management

Breztri/Trixeo **THARROS**[#] (LABA/LAMA/ICS) cardiopulmonary outcomes trial in COPD

Ultomiris (anti-complement C5 mAb)
ICAN immunoglobulin A nephropathy

New to registration

NME

acoramidis[#] (oral TTR stabilizer)
transthyretin amyloid cardiomyopathy

datopotamab deruxtecan (TROP2 ADC)
TROPION-Lung01[#] NSCLC with or without actionable genomic alterations (2L+)

Additional indication

datopotamab deruxtecan (TROP2 ADC)
TROPION-Breast01[#] HR+ HER2- breast cancer (2L/3L)

As of 25 April 2024.
Appendix: Glossary.

Project movements since Q4 2023 update

Removed from Phase I

NME
AZD5055 (porcupine inhibitor)
idiopathic pulmonary fibrosis

AZD7503 (ASO) non-alcoholic steatohepatitis

Removed from Phase II

NME
MEDI6570# (LOX-1 mAb) cardiovascular disease

Additional indication
vemircopan (oral factor D inhibitor)
generalised myasthenia gravis

Removed from Phase III

Approved/removed from registration

Life-cycle management
Enhertu (HER2-targeting ADC)
DESTINY-PanTumour02# HER2-expressing solid tumours

***Tagrisso* + CTx** (EGFR inhibitor + CTx)
FLAURA2 advanced EGFRm NSCLC (1L)

As of 25 April 2024.
Appendix: Glossary.

Q1 2024 Oncology new molecular entity[1] pipeline

Phase I

18 New Molecular Entities

- AZD0305 GPRC5D ADC) relapsed/refractory multiple myeloma
- AZD0486 (CD19-CD3 TCE) B-cell acute lymphoblastic leukemia
- AZD1390 (ATM inhibitor) glioblastoma
- AZD5335 (anti-FRα TOP1i ADC) ovarian cancer, lung adenocarcinoma
- AZD5863 (CLDN18.2 x CD3 bi-specific antibody) solid tumours
- AZD8421 (CDK2 inhibitor) solid tumours
- AZD9829 (CD123 TOP1i ADC) AML, MDS
- AZD0120 (autologous anti-CD19 and anti-BCMA CART cell immunotherapy) multiple myeloma
- volrustomig + lenvatinib (PD-1/CTLA-4+VEGF) advanced RCC
- AZD0486 (CD19-CD3 TCE) R/R B-cell non-Hodgkin lymphoma
- AZD0754 (STEAP2 CAR-T) prostate cancer
- AZD3470 (PRMT5 inhibitor) cHL
- AZD5851 (GPC3 CAR-T) hepatocellular carcinoma
- AZD6422 CLDN18.2 CAR-T solid tumours
- AZD9592 (EGFR/cMET) solid tumours
- NT-112# (TGFBR2 KO armoured TCR-T targeting KRAS G12D) solid tumours
- NT-175# (TGFBR2 KO Armored TCR-T targeting TP53 R175H) solid tumours
- NT-125# (autologous, fully-individualised, multi-specific TCR therapy targeting neoantigens) solid tumours

Under review

2 New Molecular Entities

- datopotamab deruxtecan (TROP2 ADC 2L/3L) TROPION-Breast01# HR+ HER2- breast cancer
- datopotamab deruxtecan (TROP2 ADC) TROPION-Lung01# NSCLC with or without actionable genomic alterations (2L+)

Phase II

11 New Molecular Entities

- AZD0171 + *Imfinzi* + CTx (anti-LIF + PD-L1 + CTx) metastatic PDAC (1L)
- AZD0901 (CLDN18.2 MMAE ADC) solid tumours
- AZD8205 (B7-H4 targeting ADC) solid tumours
- AZD9574 (PARP inhibitor) advanced solid malignancies
- camizestrant (SERD) HR+ breast cancer
- ceralasertib (ATR) solid tumours
- IPH5201 + *Imfinzi*# (CD39 + PD-L1) neoadjuvant/adjuvant NSCLC
- rilvegostomig (PD-1/TIGIT bispecific mAb) ARTEMIDE-01# solid tumours
- sabestomig (PD-1/TIM3 bispecific mAb) solid tumours, haematological malignancies
- saruparib (PARP1-sel) solid tumours
- volrustomig (PD-1/CTLA-4) solid tumours

Phase III

21 New Molecular Entities

- AZD0901 (CLDN18.2 MMAE ADC) CLARITY-Gastric01 gastric 2L+
- camizestrant + palbociclib (SERD+CDK4/6) SERENA-4 HR+ HER2- breast cancer (1L)
- camizestrant (selective estrogen receptor degrader) CAMBRIA-2 ER+/HER2- early breast cancer
- datopotamab deruxtecan (TROP 2 ADC) AVANZAR# NSCLC, squamous and non-squamous NSCLC, TROP2 BM+ (1L)
- datopotamab deruxtecan (TROP2 ADC) TROPION-Breast03# adjuvant residual TNBC
- datopotamab deruxtecan (TROP2 ADC) TROPION-Breast05# PD-L1+ TNBC (1L)
- datopotamab deruxtecan (TROP2 ADC) TROPION-Lung08# metastatic NSCLC (1L)
- *Imfinzi* +/- oleclumab +/- monalizumab (PD-L1 + NKG2A or PD-L1 + CD73) PACIFIC-9# unresectable Stage III NSCLC
- saruparib (PARP1-sel) EvoPAR-Prostate01 metastatic castration-sensitive prostate cancer
- volrustomig (PD-1/CTLA-4 bispecific mAb) eVOLVE-HNSCC unresected locally advanced HNSCC
- volrustomig (PD-1/CTLA-4 bispecific mAb) eVOLVE-Meso unresectable malignant pleural mesothelioma (1L)
- camizestrant + CDK4/6i (SERD + CDK4/6) SERENA-6 HR+ HER2- ESR1m breast cancer (1L)
- camizestrant (SERD) CAMBRIA-1 HR+ HER2- extended adjuvant breast cancer
- ceralasertib + *Imfinzi* (ATR inhibitor + PDL-1) LATIFY NSCLC
- datopotamab deruxtecan (TROP2 ADC) TROPION-Breast02# TNBC (1L)
- datopotamab deruxtecan (TROP2 ADC) TROPION-Breast04# neoadjuvant/adjuvant triple negative or HR-low/HER2-negative breast cancer
- datopotamab deruxtecan (TROP2 ADC) TROPION-Lung07# NSCLC PD-L1 <50% non-squamous (1L)
- datopotamab deruxtecan (TROP2 ADC) TROPION-Lung10# locally adv./met. non-squamous NSCLC with high PD-L1 expression and without actionable genomic alterations
- Rilvegostomig (PD-1/TIGIT bispecific mAb) ARTEMIDE-Biliary01# adjuvant biliary tract cancer
- volrustomig (PD-1/CTLA-4 bispecific mAb) eVOLVE-Cervical locally advanced cervical cancer
- volrustomig (PD-1/CTLA-4 bispecific mAb) eVOLVE-Lung02 metastatic NSCLC (1L)

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 ¶ Registrational Phase II trial
As of 25 April 2024.
Appendix: Glossary.

● Precision medicine approach being explored

Q1 2024 Oncology lifecycle management[1] pipeline

Phase I
1 Project

- *Enhertu* (platform) (HER2 ADC)
  DESTINY-Breast08# HER2-low breast cancer

Phase II
9 Projects

- *Enhertu* (platform) (HER2 ADC)
  DESTINY-Breast07# HER2+ breast cancer
- *Enhertu* (HER2 ADC)
  DESTINY-PanTumour01# HER2-mutant tumours
- *Imfinzi* (platform) (PD-L1 1L)
  BEGONIA metastatic TNBC
- *Imfinzi* (platform) (PD-L1 + multiple novel therapies)
  HUDSON post-IO NSCLC
- *Imfinzi* (platform) (PD-L1 mAb + multiple novel therapies) NeoCOAST-2# NSCLC
- *Lynparza* (basket) (PARP)
  LYNK002# HRRm cancer
- *Tagrisso* + *Orpathys* (EGFR + MET)
  SAVANNAH# advanced EGFRm NSCLC
- *Tagrisso* (platform) (EGFR + multiple novel therapies)
  ORCHARD# EGFRm osimertinib-resistant NSCLC (2L)
- *Truqap* (AKT)
  prostate cancer

Phase III
33 Projects

- *Calquence* + R-CHOP (BTK + R-CHOP)
  ESCALADE DLBCL (1L)
- *Enhertu* (HER2 ADC)
  DESTINY-Breast05# HER2+ post-neoadjuvant high-risk breast cancer
- *Enhertu* (HER2 ADC)
  DESTINY-Breast09# HER2+ breast cancer (1L)
- *Imfinzi* + CRT (PD-L1 + CRT)
  PACIFIC-5 (China)# locally-advanced Stage III NSCLC
- *Imfinzi* + domvanalimab (PD-L1 + TIGIT + CTx)
  PACIFIC-8# unresectable stage III NSCLC
- *Imfinzi* + Imjudo + TACE +/- lenvatinib (PD-L1 + CTLA4 + VEGF +/- chemoembolization)
  EMERALD-3 locoregional HCC
- *Imfinzi* + VEGF (PD-L1 + VEGF)
  EMERALD-2# adjuvant HCC
- Lynparza + *Imfinzi* + bevacizumab (PARP + PD-L1 + VEGF) DUO-O# ovarian cancer (1L)
- *Orpathys* + *Imfinzi* (MET + PD-L1)
  SAMETA# papillary renal cell carcinoma (1L)
- *Tagrisso* (EGFR)
  ADAURA2 adjuvant EGFRm NSCLC Stage Ia2-Ia3 following complete tumour resection
- *Truqap* + CTx (AKT + chemotherapy)
  CAPItello-290 mTNBC (1L)
- *Calquence* + venetoclax + obinutuzumab (BTK + BCL-2 + anti-CD20) AMPLIFY# CLL (1L)
- *Enhertu* (HER2 ADC)
  DESTINY-Breast11# neoadjuvant HER2+ breast cancer
- *Enhertu* (HER2 ADC)
  DESTINY-Gastric04# HER2+ gastric cancer (2L)
- *Imfinzi* + CRT (PD-L1 + CRT)
  KUNLUN# locally-advanced ESCC
- *Imfinzi* + EV +/- *Imjudo* (PD-L1 + nectin-4 targeting ADC +/- CTLA-4) VOLGA MIBC
- *Imfinzi* + *Imjudo* + SoC (PD-L1 + CTLA-4 + SoC)
  NILE urothelial cancer (1L)
- *Imfinzi* +/- *Imjudo* + CRT (PD-L1 +/- CTLA-4 + CRT)
  ADRIATIC# LS-SCLC (1L)
- *Imfinzi* (PD-L1)
  POTOMAC non-muscle invasive bladder cancer
- *Tagrisso* (EGFR inhibitor)
  LAURA Stage III EGFRm NSCLC
- *Tagrisso* +/- CTx neoadjuvant (EGFR +/- CTx)
  NeoADAURA Stage II/III resectable EGFRm NSCLC
- *Truqap* + docetaxel (AKT + docetaxel)
  CAPItello-280 mCRPC prostate cancer
- *Calquence* (BTK inhibitor)
  ECHO# MCL (1L)
- *Enhertu* (HER2 ADC)
  DESTINY-Breast06# post-ET HER2-low/HR+ breast cancer (2L)
- *Enhertu* (HER2 ADC)
  DESTINY-Lung04# HER2m NSCLC (1L)
- *Imfinzi* + CTx (PD-L1 + CTx)
  NIAGARA muscle invasive bladder cancer
- *Imfinzi* + FLOT (PD-L1 + CTx)
  MATTERHORN# neoadjuvant/adjuvant gastric cancer
- *Imfinzi* + VEGF + TACE (PD-L1 + VEGF + TACE)
  EMERALD-1# locoregional HCC
- *Imfinzi* post-SBRT (PD-L1 mAb)
  PACIFIC-4# post-SBRT Stage I/II NSCLC
- *Lynparza* (PARP)
  MONO-OLA1# BRCAwt ovarian cancer (1L)
- *Tagrisso* + *Orpathys* (EGFR + MET)
  SAFFRON# advanced EGFRm NSCLC
- *Truqap* + abiraterone (AKT + abiraterone)
  CAPItello-281 PTEN deficient mHSPC
- *Truqap* + *Faslodex* + palbociclib (AKT + fulvestrant + CDK4/6) CAPItello-292 triplet in early relapse/ET resistant locally advanced or mBC (1L)

Under review
2 Projects

- *Imfinzi* + CTx neoadjuvant (PD-L1 + CTx)
  AEGEAN locally-advanced Stage II-III NSCLC
- Lynparza + *Imfinzi* (PARP + PD-L1)
  DUO-E# endometrial cancer (1L)

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 ¶ Registrational Phase I/III trial
As of 25 April 2024.
Appendix: Glossary.

● Precision medicine approach being explored

Q1 2024 BioPharmaceuticals new molecular entity[1] pipeline

Phase I
17 New Molecular Entities

- AZD1163 (bispecific antibody) rheumatoid arthritis
- AZD0292 (pseudomonas PsI-PcrV bispecific mAb) non-CF bronchiectasis
- AZD2373 (podocyte) health nephropathy
- AZD4041# (orexin 1 receptor antagonist) opioid use disorder
- AZD6234 (peptide) obesity with related comorbidities
- AZD6912 (siRNA) rheumatoid arthritis
- AZD8630# (inhaled TSLP FAb) asthma
- COVID mRNA VLP vaccine (vaccine) COVID-19
- MEDI1814# (amyloid beta mAb) Alzheimer's disease
- AZD1705 lipid lowering cardiovascular disease
- AZD2389 (anti-fibrotic mechanism) MASH
- AZD4144 (inflammation modulator) cardiorenal disease
- AZD5462# (RXFP1 agonist) heart failure
- AZD6793 (IRAK4 inhibitor) inflammatory diseases
- AZD7798 (humanised mAb targeting T-cells subset) Crohn's disease
- AZD9550 (GLP-1R glucagon dual agonist) MASH
- MEDI0618* (PAR2 antagonist) migraine

Phase II
11 New Molecular Entities

- atuliflapon (FLAP) asthma
- AZD0780 (PCSK9) dyslipidemia
- AZD2693 MASH
- AZD3427 (relaxin) mimetic heart failure
- AZD4604 (inhaled JAK1 inhibitor) asthma
- MEDI1341# (alpha synuclein mAb) multiple system atrophy/Parkinson's disease
- MEDI7352 (NGF/TNF) Osteoarthritis pain, PDN
- mitiperstat (MPO) HFpEF, MASH
- mitiperstat (myeloperoxidase) COPD
- tozorakimab (IL-33) FRONTIER-3 asthma
- zibotentan/dapagliflozin (endothelin A receptor antagonist/SGLT2i) liver cirrhosis

Phase III
7 New Molecular Entities

- balcinrenone/dapagliflozin (MR + SGLT2i) heart failure with CKD
- baxdrostat (aldosterone synthase inhibitor) hypertension
- baxdrostat/dapagliflozin (aldosterone synthase inhibitor and reversible SGLT2i) CKD
- sipavibart SUPERNOVA¶ (SARS-CoV-2 LAAB) prevention of COVID-19
- tozorakimab (IL-33) OBERON TITANIA PROSPERO MIRANDA COPD
- tozorakimab (IL-33) TILIA 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 * Phase I/IIa ¶ Registrational Phase I/III trial
As of 25 April 2024.
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● Precision medicine approach being explored

Q1 2024 BioPharmaceuticals life cycle management[1] pipeline

Phase I
0 Projects

Phase II
2 Projects

- *Andexxa* (anti-factor Xa reversal)
  urgent surgery
- *Tezspire* (TSLP)
  COURSE# COPD

Phase III
12 Projects

- *Breztri/Trixeo* (LABA/LAMA/ICS)
  THARROS# cardiopulmonary outcomes trial in COPD
- *Breztri/Trixeo* (ABA/LAMA/ICS)
  KALOS LOGOS asthma
- *Breztri/Trixeo* (LABA/LAMA/ICS)
  ATHLOS COPD cardiopulmonary exercise trial
- *Fasenra* (IL-5R)
  RESOLUTE# chronic obstructive pulmonary disease
- *Fasenra* (IL-5R)
  NATRON hypereosinophilic syndrome
- *Fasenra* (IL-5R)
  ORCHID# nasal polyps
- *Saphnelo* (type I IFN receptor mAb)
  DAISY# systemic sclerosis
- *Saphnelo* (type I IFN receptor mAb)
  IRIS# lupus nephritis
- *Saphnelo* (type I IFN receptor mAb)
  TULIP-SC# SLE s.c.
- *Tezspire* (TSLP)
  WAYPOINT# nasal polyps
- *Tezspire* (TSLP)
  CROSSING# eosinophilic esophagitis
- *Wainua*# (LICA)
  CARDIO-TTRansform ATTR-CM

Under review
2 Projects

- *Fasenra* (IL-5R)
  MANDARA eosinophilic granulomatosis with polyangiitis
- roxadustat# (HIFPH)
  chemotherapy-induced anaemia

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 ¶ Registrational Phase I/III trial
As of 25 April 2024.
Appendix: Glossary.

Precision medicine approach being explored

Q1 2024 Rare Disease pipeline[1]

Phase I
4 Projects

- ALXN1910 (next-generation TNSALP ERT)
  bone metabolism
- ALXN1920 (kidney-targeted factor H fusion protein)
  nephrology
- ALXN2030 (siRNA targeting complement C3)
  nephrology
- ALXN2080 (oral factor D)
  healthy volunteers

Phase II
3 Project

- danicopan (factor D)
  geographic atrophy
- *Ultomiris* (anti-complement C5 mAb)
  proliferative lupus nephritis
- vemircopan (oral factor D)
  proliferative lupus nephritis or immunoglobulin A nephropathy

Phase III
7 Projects

- ALXN2220# (TTR depleter)
  DepleTTR-CM ATTR-CM
- anselamimab (fibril-reactive mAb)
  AL amyloidosis
- efzimfotase alfa (ALXN1850) (next-generation TNSALP ERT)
  HICKORY CHESTNUT hypophosphatasia
- gefurulimab (humanised bispecific V_HH antibody)
  generalised myasthenia gravis
- *Ultomiris* (anti-complement C5 mAb)
  haematopoietic stem cell transplant–associated thrombotic microangiopathy
- *Ultomiris* (anti-complement C5 mAb)
  ICAN immunoglobulin A nephropathy
- *Ultomiris* (anti-complement C5 mAb)
  ARTEMIS cardiac surgery-associated acute kidney injury

Under review
1 Project

- acoramidis# (oral TTR stabiliser)
  ATTR-CM

Phase progressions based on first subject in achievement
1. Includes new molecular entities and significant lifecycle management projects
Partnered and/or in collaboration ¶ Registrational Phase II/III trial
As of 25 April 2024.
Appendix: Glossary.

● Precision medicine approach being explored

Designations in our pipeline

3 Accelerated approvals

- *Andexxa* acute major bleed (US)
- *Beyfortus* RSV mAb-YTE (EU)
- *Calquence* MCL (1L) (US)

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

- *Beyfortus* RSV mAb-YTE MELODY-MEDLEY (US)
- *Beyfortus* RSV mAb-YTE MELODY-MEDLEY (CN)
- *Beyfortus* RSV mAb-YTE MELODY-MEDLEY (EU)[1]
- *Tezspire* asthma NAVIGATOR (US)
- tozorakimab severe viral LRTD TILIA (CN)
- *Calquence* MCL (1L) (US)
- *Enhertu* HER2-overexpressing tumors DESTINY-PanTumor02 (US)
- *Enhertu* HER2+/HER2-low gastric (3L) DESTINY-Gastric01 (US)
- *Enhertu* HER2+/HER2-low gastric (3L) DESTINY-Gastric01 (JP)[2]
- *Enhertu* HER2m NSCLC (2L+) DESTINY-Lung01 (US)
- *Tagrisso* + CTx EGFRm NSCLC (1L) FLAURA2 (US)
- *Voydeya* PNH-EVH (US)
- *Voydeya* PNH-EVH (EU)

9 Fast Track

- AZD3427 relaxin mimetic heart failure (US)
- *Beyfortus* RSV mAb-YTE MELODY-MEDLEY (US)
- *Saphnelo* SLE (US)
- tozorakimab severe viral LRTD TILIA (US)
- *Wainua* ATTR-Cardiomyopathy (US)
- camizestrant 1L HR+ HER2- ESR1m breast cancer SERENA-6 (US)
- *Orpathys* + *Tagrisso* NSCLC SAVANNAH/SAFFRON (US)
- *Truqap* + fulv HR+ breast (2L+) CAPItello-291 (US)
- anselamibab AL amyloidosis (US)

12 Priority Review

- *Beyfortus* RSV mAb-YTE MELODY-MEDLEY (CN)
- *Roxadustat* chronic kidney disease (CN)
- *Tezspire* asthma NAVIGATOR (US)
- *Calquence* MCL (1L) (US)
- *Enhertu* HER2 overexpressing tumors DESTINY-PanTumor02 (US)
- *Enhertu* HER2+/HER2-low gastric (3L) DESTINY-Gastric01 (US)
- *Imfinzi* + *Imjudo* HCC (1L) HIMALAYA (US)
- *Lynparza* + abiraterone all-comers mCRPC (1L) PROpel (US)
- *Lynparza* gBRCA adjuvant breast OlympiA (US)
- *Tagrisso* + CTx EGFRm NSCLC (1L) FLAURA2 (US)
- *Truqap* + fulv HR+ breast (2L+) CAPItello-291 (US)
- *Ultomiris* gMG (US)

21 Orphan

- *Andexxa* acute major bleed (JP)
- *Fasenra* EGPA MANDARA (US)
- *Fasenra* HES NATRON (US)
- *Saphnelo* systemic sclerosis (US)
- *Tezspire* EoE CROSSING (US)
- *Wainua* transthyretin-mediated amyloidosis (EU)
- *Wainua* transthyretin-mediated amyloidosis (US)
- *Calquence* CLL (1L) (US)
- *Calquence* CLL (1L) (EU)
- *Calquence* MCL (1L) (US)
- *Enhertu* HER2+/HER2-low gastric (3L) DESTINY-Gastric01 (US)
- *Imfinzi* +/- *Imjudo* HCC (1L) HIMALAYA (EU)
- *Imfinzi* +/- *Imjudo* HCC (1L) HIMALAYA (US)
- *Lynparza* gBRCA adjuvant breast OlympiA (JP)
- ALXN2220 ATTR-CM (US)
- anselamibab AL amyloidosis (US)
- anselamibab AL amyloidosis (EU)
- gefurulimab myasthenia gravis (US)
- *Ultomiris* HSCT-TMA (US)
- *Voydeya* PNH (US)
- *Voydeya* PNH (EU)

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

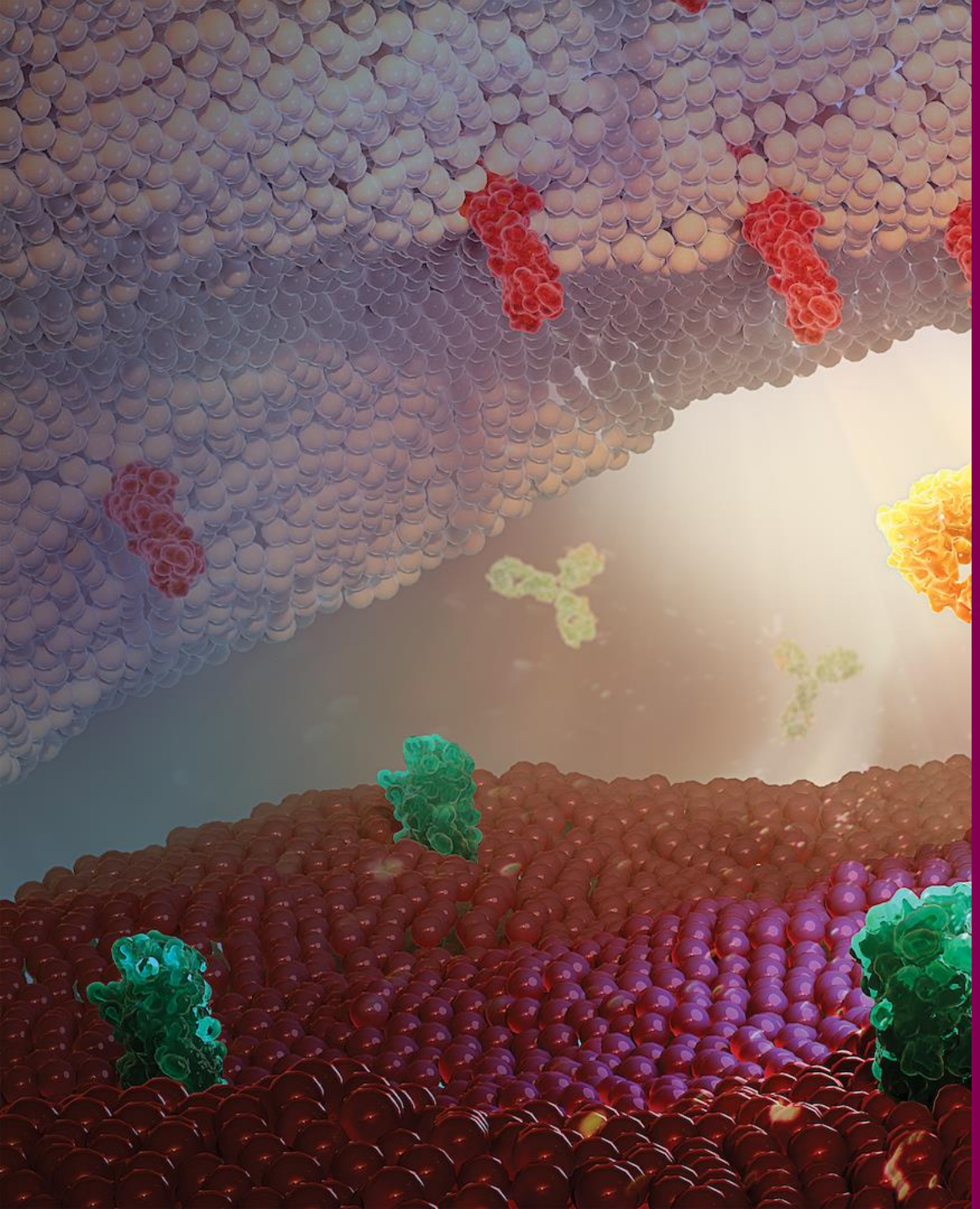
FAST TRACK is a process designed to facilitate the development, and expedite the review of medicines to treat serious conditions and fill an unmet medical need

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

NOTE: excludes designations for projects which have launched in all applicable major markets
As of 25 April 2024.
Appendix: Glossary.

Oncology BioPharmaceuticals Rare Disease



Oncology:
approved medicines and late-stage pipeline

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 • Global trial – 17 countries	• 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 • Data anticipated: >2025
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* • Global trial – 23 countries	• Primary endpoint: mPR • Secondary endpoints: cPR, EFS, DFS and OS	• FPCD: Q1 2021 • LPCD: Q4 2023 • Data anticipated: H2 2024
Phase III FLAURA2 NCT04035486	1L EGFRm NSCLC	586	• Arm 1: *Tagrisso* + pemetrexed/carboplatin or pemetrexed/cisplatin • Arm 2: *Tagrisso* • Global trial – 23 countries	• Primary endpoint: PFS • Secondary endpoints: OS, LOS, ORR DoR, depth of response, PFS2, QoL and PK parameters	• FPCD: Q4 2019 • Data readout: Q2 2023 • Primary endpoint met

As of 25 April 2024.
Appendix: Glossary.

Tagrisso (highly-selective, irreversible EGFR inhibitor)

NSCLC, combinations

Trial	Population	Patients	Design	Endpoints	Status
Phase III **SAFFRON** **NCT05261399** **Partnered (HUTCHMED)**	EGFR-mutated, 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: 2025
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 2024
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	• Primary endpoint: PFS	• FPCD: Q3 2021 • Data anticipated: H2 2024
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* • Global trial	• Primary endpoint: ORR • Secondary endpoints: PFS, DoR and OS	• FPCD: Q1 2019 • Data anticipated: H2 2024 • Initial data readout: Q2 2020
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 • Global trial – 9 countries	• Primary endpoint: ORR • Secondary endpoints: PFS, DoR, OS, safety and tolerability	• FPCD: Q3 2019 • LPCD: Q4 2023 • Data anticipated: 2025

As of 25 April 2024.
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: >2025
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: >2025
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 anticipated: 2025
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 TOPAZ-1 NCT03875235	1L BTC	810	• Arm 1: *Imfinzi* + gemcitabine + cisplatin • Arm 2: placebo + gemcitabine + cisplatin • Global trial	• Primary endpoint: OS • Secondary endpoints: PFS, ORR and DoR	• FPCD: Q2 2019 • LPCD: Q4 2020 • 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: >2025

As of 25 April 2024.
Appendix: Glossary.

Imfinzi (PD-L1 mAb)

Lung cancer

Trial	Population	Patients	Design	Endpoints	Status
Phase III AEGEAN NCT03800134	Neoadjuvant NSCLC patients, Stage II and III resected NSCLC (incl. EGFR/ALK-positive)	800	• 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)	1360	• 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 anticipated: H1 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: >2025
Phase III PACIFIC-5 NCT03706690	Unresected, locally advanced NSCLC	360	• 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 • Data anticipated: H2 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: >2025
Phase III ADRIATIC NCT03703297	Limited-stage SCLC 1L following platinum-based concurrent chemoradiation therapy	600	• 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

As of 25 April 2024.
Appendix: Glossary.

Imfinzi (PD-L1 mAb)

Lung cancer

Trial	Population	Patients	Design	Endpoints	Status
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: >2025
Phase II HUDSON NCT03334617	NSCLC, patients who progressed on an anti-PD-1/PD-L1-containing therapy	529	• 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 anticipated: H2 2024
Phase II NeoCOAST-2 NCT05061550	Early-stage, resectable NSCLC (Stage II to Stage IIIA)	490	• 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: datopotamab deruxtecan + single agent platinum chemotherapy • Arm 5: AZD0171 + platinum doublet chemotherapy	• Primary endpoints: pCR, safety	• FPCD: Q2 2022 • Data anticipated: >2025
Phase I/II SCope-D1 NCT04870112	NSCLC, SCLC	18	• Open-label, multi-centre trial • s.c. *Imfinzi*	• Primary endpoints: PK parameters and safety	• FPCD: Q4 2021 • LPCD: Q2 2022 • Data anticipated: H2 2024

As of 25 April 2024.
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 anticipated: 2025
Phase III NIAGARA NCT03732677	Muscle-invasive bladder cancer	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 anticipated: 2025
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: 2025
Phase III NILE NCT03682068	1L bladder cancer	1244	• Arm 1: *Imfinzi* + *Imjudo* + SoC • Arm 2: *Imfinzi* + SoC • Arm 3: SoC	• Primary endpoint: OS	• FPCD: Q4 2018 • LPCD: Q2 2021 • Data anticipated: H2 2024
Phase III VOLGA NCT04960709	Muscle-invasive bladder cancer ineligible to cisplatin	830	• 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 • Data anticipated: 2025
Phase II BEGONIA NCT03742102	1L mTNBC	240	• Arm 1: *Imfinzi* + paclitaxel • Arm 2: *Imfinzi* + paclitaxel + *Truqap* • Arm 5: *Imfinzi* + paclitaxel + oleclumab • Arm 6: *Imfinzi* + *Enhertu* • Arm 7: *Imfinzi* + datopotamab deruxtecan • Arm 8: *Imfinzi* + datopotamab deruxtecan (PD-L1-high) • Global trial	• Primary endpoints: safety and tolerability • Secondary endpoints: ORR, PFS, DoR, OS, PK and ADA	• FPCD: Q1 2019 • Data anticipated: 2025

As of 25 April 2024.
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 25 April 2024.
Appendix: Glossary.

Lynparza (PARP inhibitor)

Multiple cancers

Trial	Population	Patients	Design	Endpoints	Status
Phase III OlympiA NCT02032823 Partnered (BIG & NRG Oncology)	gBRCAm adjuvant breast cancer	1836	• Arm 1: *Lynparza* BID 12-month duration • Arm 2: placebo 12-month duration • Global trial in partnership with Breast International Group and National Cancer Institute/NRG Oncology	• Primary endpoint: iDFS • Secondary endpoints: distant disease-free survival and OS	• FPCD: Q2 2014 • LPCD: Q2 2019 • Data readout: Q1 2021 • Primary endpoint met
Phase III MONO-OLA1 NCT04884360	BRCAwt advanced ovarian cancer, 1L maintenance	420	• 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 • Data anticipated: H2 2024
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 endpoints: OS	• FPCD: Q4 2018 • LPCD: Q3 2022 • Data readout: Q3 2021 • Primary endpoint met

As of 25 April 2024.
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	• Primacy 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: 2025
Phase III DESTINY-Breast06 NCT04494425 Partnered (Daiichi Sankyo)	HER2-low, HR+ breast cancer with disease progression on endocrine therapy in the metastatic setting	850	• 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 • Data anticipated: H1 2024
Phase III DESTINY-Breast09 NCT04784715 Partnered (Daiichi Sankyo)	HER2-positive, metastatic breast cancer with no prior therapy for advanced or metastatic disease	1134	• 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 anticipated: 2025

As of 25 April 2024.
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	900	• 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 anticipated: 2025
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: 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 • Data readout: Q3 2023

As of 25 April 2024.
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 gastro-esophageal junction 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 anticipated: 2025
Phase II **DESTINY-Gastric06** **NCT04989816** **Partnered (Daiichi Sankyo)**	HER2-positive gastric cancer or gastro-esophageal junction 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)**	HER2-overexpressing gastric or gastroeosophogeal junction cancer	357	• 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 durvalumab • Part 2: 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 3A: *Enhertu*, FP and volrustomig • Arm 3B: *Enhertu*, FP and volrustomig • Arm 4A: *Enhertu*, FP and rilvegostomig • Arm 4B: *Enhertu*, FP and rilvegostomig	• Primary endpoint (Part 1): safety • Primary endpoint (Part 2): ORR • Secondary endpoints: DoR, DCR, PFS, OS, PK parameters and presence of ADAs	• FPCD: Q2 2020 • Data anticipated: >2025

As of 25 April 2024.
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	264	• 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: 2025
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 • 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	168	• 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)	• Primary endpoint: safety and RP2D • Secondary endpoints: ORR, DoR, DCR, PFS, OS and PK parameters	• FPCD: Q4 2021 • Data anticipated: >2025

As of 25 April 2024.
Appendix: Glossary.

Enhertu (trastuzumab deruxtecan, HER2 ADC)

Other cancers

Trial	Population	Patients	Design	Endpoints	Status
Phase Ib U106 NCT04042701 Partnered (Daiichi Sankyo)	HER2-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 2024

As of 25 April 2024.
Appendix: Glossary.

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 • Data anticipated: 2025
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 • Data anticipated: 2025
Phase III ESCALADE NCT04529772	DLBCL	600	• *Calquence* + rituximab, cyclophosphamide, doxorubicin, vincristine and prednisone	• Primary endpoint: PFS	• FPCD: Q1 2021 • Data anticipated: >2025
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 anticipated: H1 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: >2025
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 anticipated: H1 2024

As of 25 April 2024.
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 • Data anticipated: H2 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 2024

As of 25 April 2024.
Appendix: Glossary.

Truqap (capivasertib, AKT inhibitor)

Breast cancer and prostate cancer

Trial	Population	Patients	Design	Endpoints	Status
Phase III CAPItello-290 NCT03997123	Locally advanced or metastatic TNBC	924	• Double-blind, randomised, comparative trial • Arm 1: *Truqap* + paclitaxel • Arm 2: placebo + paclitaxel	• Primary endpoint: OS	• FPCD: Q3 2019 • LPCD: Q1 2022 • Data anticipated: H1 2024
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	1000	• Double-blind, randomised, comparative trial • Arm 1: *Truqap* + abiraterone • Arm 2: placebo + abiraterone	• Primary endpoint: rPFS	• FPCD: Q3 2020 • Data anticipated: H2 2024
Phase III CAPItello-280 NCT05348577	mCRPC prostate cancer	790	• Double-blind, randomised, comparative trial • Arm 1: *Truqap* + docetaxel • Arm 2: placebo + docetaxel	• Primary endpoint: OS	• FPCD: Q2 2022 • Data anticipated: >2025
Phase Ib/III CAPItello-292 NCT04862663	1L triplet in early relapse/endocrine-resistant locally advanced (inoperable) or metastatic HR+/HER2-negative breast cancer	700	• Double-blind, randomised, comparative trial • Arm 1: *Truqap* + palbociclib + *Faslodex* • Arm 2: placebo + palbociclib + *Faslodex*	• Primary endpoint: PFS	• FPCD: Q2 2021 • Data anticipated: >2025

As of 25 April 2024.
Appendix: Glossary.

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	733	• Open-label, randomised trial • Arm 1: datopotamab deruxtecan • 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
Phase III TROPION-Breast02 NCT05374512 Partnered (Daiichi Sankyo)	Locally recurrent inoperable or metastatic TNBC	600	• Open-label, randomised trial • Arm 1: datopotamab deruxtecan • Arm 2: investigator's choice of chemotherapy (paclitaxel, nab-paclitaxel, carboplatin, capecitabine, eribulin mesylate) • Global trial	• Primary endpoints: PFS (BICR) and OS • Secondary endpoints: PFS (Inv), ORR, DoR, PK parameters and ADA	• FPCD: Q2 2022 • Data anticipated: H2 2024
Phase III TROPION-Breast03 NCT05629585 Partnered (Daiichi Sankyo)	Stage I-III TNBC without pathological complete response following neoadjuvant therapy	1075	• Open-label, randomised • Arm 1: datopotamab deruxtecan + *Imfinzi* • Arm 2: datopotamab deruxtecan • Arm 3: investigator's choice of therapy (capecitabine, pembrolizumab, or capecitabine + pembrolizumab) • Global trial	• Primary endpoint: iDFS • Secondary endpoints: DDFS, OS, PK and ADA	• FPCD: Q4 2022 • Data anticipated: >2025
Phase III TROPION-Breast04 NCT06112379 Partnered (Daiichi Sankyo)	Neoadjuvant/adjuvant triple-negative or HR-low/HER2-negative breast cancer	1728	• Open-label, randomised • Arm 1: datopotamab deruxtecan + durvalumab • Arm 2: pembrolizumab + chemotherapy • Global trial	• Dual primary endpoint: pCR and EFS • Secondary endpoints: OS, DDFS and safety	• FPCD: Q4 2023 • Data anticipated: >2025
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: datopotamab deruxtecan + durvalumab • Arm 2: investigator's choice of chemotherapy in combination with pembrolizumab (paclitaxel, nab-paclitaxel, or gemcitabine + carboplatin) • Arm 3: datopotamab deruxtecan • Global trial	• Primary endpoint: PFS (BICR) • Secondary endpoint: OS, PFS (inv), ORR, DoR, DCR and safety	• FPCD: Q4 2023 • Data anticipated: >2025

As of 25 April 2024.
Appendix: Glossary.

datopotamab deruxtecan (TROP2 ADC)

NSCLC

Trial	Population	Patients	Design	Endpoints	Status
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: datopotamab deruxtecan • Arm 2: docetaxel • Global trial	• 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: datopotamab deruxtecan + pembrolizumab • Arm 2: pembrolizumab • Global trial	• Primary endpoints: PFS and OS	• FPCD: Q1 2022 • Data anticipated: >2025
Phase III TROPION-Lung07 NCT05555732 Partnered (Daiichi Sankyo)	1L patients with PD-L1 TPS <50% and advanced or metastatic NSCLC without actionable genomic alternations	975	• Randomised, open-label • Arm 1: datopotamab deruxtecan + pembrolizumab + platinum chemotherapy • Arm 2: datopotamab deruxtecan + pembrolizumab • Arm 3: pembrolizumab + pemetrexed + platinum chemotherapy • Global trial	• Primary endpoints: PFS and OS	• FPCD: Q1 2023 • Data anticipated: >2025
Phase III AVANZAR NCT05687266	1L NSCLC	1280	• Arm 1: carboplatin + datopotamab deruxtecan + *Imfinzi* • Arm 2: pembrolizumab • Global trial	• Co-primary endpoints: OS and PFS in TROP2 biomarker-positive	• FPCD: Q1 2023 • Data anticipated: 2025
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: datopotamab deruxtecan in combination with rilvegostomig • Arm 2: rilvegostomig monotherapy • Arm 3: pembrolizumab monotherapy	• Primary endpoints: PFS and OS in TROP2 biomarker-positive participants • Secondary endpoints: PFS and OS in the ITT population, ORR, DoR, TTD, PK, immunogenicity and PFS2	• Initiating • Data anticipated: >2025

As of 25 April 2024.
Appendix: Glossary.

datopotamab deruxtecan (TROP2 ADC)

NSCLC

Trial	Population	Patients	Design	Endpoints	Status
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 • datopotamab deruxtecan • Global trial	• Primary endpoint: ORR • Secondary endpoints: DOR, PFS, OS, safety, PK parameters and ADA	• FPCD: Q1 2021 • LPCD: Q1 2022 • Data anticipated: H2 2024
Phase I **TROPION-Lung02** **NCT04526691** **Partnered (Daiichi Sankyo)**	Advanced or metastatic NSCLC	145	• Open-label, two-part (dose escalation and dose expansion), sequential assignment • datopotamab deruxtecan + pembrolizumab +/- platinum chemotherapy • Global trial – US, Japan, Italy, Spain and Taiwan	• Primary endpoints: DLT and safety • Secondary endpoints: ORR, DOR, PFS, OS, PK parameters and ADA	• FPCD: Q4 2020 • LPCD: Q2 2023 • Data anticipated: H2 2024
Phase I **TROPION-Lung04** **NCT04612751** **Partnered (Daiichi Sankyo)**	Advanced or metastatic NSCLC	232	• Open-label, two-part (dose escalation, dose expansion), sequential assignment • datopotamab deruxtecan + *Imfinzi* +/- platinum chemotherapy • Cohort 1 & 2: datopotamab deruxtecan + *Imfinzi* • Cohort 3 & 4: datopotamab deruxtecan + *Imfinzi* + carboplatin • Cohort 5 & 6: datopotamab deruxtecan + rilvegostomig • Cohort 7 & 8: datopotamab deruxtecan + rilvegostomig + carboplatin • Cohort 9 &10: datopotamab deruxtecan + volrustomig + carboplatin • Cohort 11: datopotamab deruxtecan + volrustomig • Cohort 12, 13 & 14: datopotamab deruxtecan + sabestomig • Global trial	• Primary endpoints: DLT and safety • Secondary endpoints: ORR, DOR, PFS, OS, PK parameters and ADA	• FPCD: Q1 2021 • Data anticipated: >2025

As of 25 April 2024.
Appendix: Glossary.

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: datopotamab deruxtecan monotherapy • Sub-study 2 (gastric cancer); • Sub-study 2a: datopotamab deruxtecan + capecitabine • Sub-study 2b: datopotamab deruxtecan + 5-fluorouracil • Sub-study 3 (mCRPC); • Sub-study 3a: datopotamab deruxtecan (post-NHA) • Sub-study 3c: datopotamab deruxtecan + prednisone/prednisolone • Sub-study 4 (ovarian cancer); • Sub-study 4a: datopotamab deruxtecan • Sub-study 4a (expansion): datopotamab deruxtecan PSR/PRR (2-3L) • Sub-study 4c: datopotamab deruxtecan + carboplatin + bevacizumab PSR (2-3L) • Sub-study 5 (CRC); • Sub-study 5a1: datopotamab deruxtecan (TROP2+ 3L+) • Sub-study 5a2: datopotamab deruxtecan (TROP2+ 2L+) • Sub-study 5b: datopotamab deruxtecan + 5-FU/leucovorin or Capecitabine + bevacizumab (TROP2+ 1L) • Sub-study 6 (bladder); • Sub-study 6d: datopotamab deruxtecan (2L+) • Sub-study 6b: 1L cis-ineligible/2L datopotamab deruxtecan + rilvegostomig (1L) • Sub-study 6c: post-pembro/EV - datopotamab deruxtecan + Carbo/Cisplatin (2L) • Sub-study 7 (BTC) • Sub-study 7a: TROP2+ datopotamab deruxtecan (2L+)	• Primary endpoints: ORR and safety	• FPCD: Q3 2022 • Data anticipated: >2025

As of 25 April 2024.
Appendix: Glossary.

datopotamab deruxtecan (TROP2 ADC)

Other cancers

Trial	Population	Patients	Design	Endpoints	Status
Phase I/II TROPION-PanTumor02 NCT05460273 Partnered (Daiichi Sankyo)	NSCLC and TNBC and other solid tumours in Chinese patients	119	• Single-arm, multi-cohort study with no blinding • datopotamab deruxtecan • China only	• Primary endpoint: ORR • Secondary endpoints: DoR, DCR, BOR, TTR PFS and OS	• FPCD: Q3 2022 • LPCD: Q2 2023 • Data readout: Q4 2023
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 • datopotamab deruxtecan • US and Japan	• Primary endpoints: DLT and safety • Secondary endpoints: PK parameters, anti-tumour activity and ADA	• FPCD: Q1 2018 • Data anticipated: H2 2024

As of 25 April 2024.
Appendix: Glossary.

camizestrant (AZD9833, next-generation oral SERD)

Breast cancer

Trial	Population	Patients	Design	Endpoints	Status
Phase III SERENA-4 NCT04711252	HR+ HER2-negative advanced breast cancer	1342	• Randomised, double-blind, comparative trial • Arm 1: camizestrant + palbociclib • Arm 2: anastrazole + palbociclib	• Primary endpoint: PFS • Secondary endpoints: OS and PFS2	• FPCD: Q1 2021 • Data anticipated: >2025
Phase III SERENA-6 NCT04964934	HR+ HER2-negative advanced breast cancer	300	• 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 • Data anticipated: 2025
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 • Global trial – 39 countries	• Primary endpoint: IBCFS • Secondary endpoints: IDFS, DRFS and OS	• FPCD: Q1 2023 • Data anticipated: >2025
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 • Global study – 43 countries	• Primary endpoint: IBCFS • Secondary endpoints: IDFS, DRFS and OS	• FPCD: Q4 2023 • Data anticipated: >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 and tolerability, PK parameters • Secondary endpoint: anti-tumour activity	• FPCD: Q1 2021 • LPCD: Q1 2023 • Data readout: Q4 2023

As of 25 April 2024.
Appendix: Glossary.

camizestrant (AZD9833, next-generation oral SERD)

Breast cancer

Trial	Population	Patients	Design	Endpoints	Status
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
Phase II SERENA-3 NCT04588298	HR+ HER2-negative early breast cancer	135	• Randomised, open-label, parallel-group, multi-centre trial • camizestrant	• Primary endpoint: change in ER expression between pre- and on-treatment tumour biopsies	• FPCD: Q4 2020 • LPCD: Q2 2023 • Data readout: Q3 2023
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 • Data anticipated: H2 2024
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 and tolerability, PK parameters • Secondary endpoint: anti-tumour activity	• FPCD: Q1 2021 • LPCD: Q1 2023 • Data readout: Q4 2023

As of 25 April 2024.
Appendix: Glossary.

ceralasertib (AZD6738, ATR inhibitor)

Multiple cancers

Trial	Population	Patients	Design	Endpoints	Status
Phase III LATIFY NCT05450692	Post-IO NSCLC	580	• Double-arm randomised: • Arm 1: ceralasertib + *Imfinzi* • Arm 2: docetaxel	• Primary endpoint: OS • Secondary endpoint: PFS, ORR, DoR, TTR, DCR, PFS2 and TTD	• FPCD: Q4 2022 • Data anticipated: 2025
Phase I/II NCT02264678	Solid tumours	466	• 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: >2025

As of 25 April 2024.
Appendix: Glossary.

rilvegostomig (AZD2936, PD-1/TIGIT bispecific mAb)

Solid tumours

Trial	Population	Patients	Design	Endpoints	Status
Phase III **ARTEMIDE-Biliary01** **NCT06109779** **Partnered (Compugen)**	BTC with curative intent	750	• Randomized, Double-Blind, Placebo-Controlled, Multicenter • Arm 1: rilvegostomig in combination with investigator's choice of chemotherapy (capecitabine, S-1 (tegafur/gimeracil/oteracil) or gemcitabine/cisplatin) • Arm 2: placebo in combination with 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: >2025
Phase I/II **ARTEMIDE-01** **NCT04995523** **Partnered (Compugen)**	NSCLC	192	• 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 • 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) • Secondary endpoints: PK parameters, PD (receptor occupancy), efficacy (DCR, DoR, DRR, PFS)	• FPCD: Q4 2021 • LPCD: Q1 2024 • Data anticipated: H2 2024

As of 25 April 2024.
Appendix: Glossary.

rilvegostomig (AZD2936, PD-1/TIGIT bispecific mAb)

Solid tumours

Trial	Population	Patients	Design	Endpoints	Status
Phase IIb GEMINI-Gastric NCT05702229 Partnered (Compugen)	Gastric cancer	240	• Open-label gastric platform study • 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: 2025
Phase IIb GEMINI-HBP NCT05775159 Partnered (Compugen)	HCC, BTC	260	• Open-label hepatobiliary platform study • 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 abd 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: 2025

As of 25 April 2024.
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: >2025
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 + datopotamab deruxtecan • Module 6: saruparib + camizestrant	• Primary endpoints: safety and tolerability, PK parameters • Secondary endpoint: efficacy	• FPCD: Q4 2020 • Data anticipated: >2025
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: >2025
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
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 endoints: safety and tolerability, impact on surgical feasibility and change in Ki67	• FPCD: Q3 2023 • Data anticipated: 2025

As of 25 April 2024.
Appendix: Glossary.

volrustomig (MEDI5752, 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	1000	• Randomised, double-blind, placebo-controlled, multi-centre trial • Arm 1: volrustomig • Arm 2: placebo	• Primary endpoint: PFS (Inv, PD-L1 expressing patients) • Secondary endpoints: PFS (Inv, ITT), OS, ORR, DoR and TFST	• FPCD: Q4 2023 • Data anticipated: >2025
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: >2025
Phase III eVOLVE-Meso NCT06097728	1L unresectable malignant pleural mesothelioma	600	• 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: >2025
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: >2025
Phase Ib NCT04522323	Advanced renal cell carcinoma	179	• Open-label, dose escalation and dose expansion trial • Arm 1: volrustomig and axitinib • Arm 2: volrustomig and 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 • Data anticipated: >2025
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 in combination with chemotherapy • Arm 1: volrustomig i.v. • Arm 2: volrustomig i.v., pemetrexed and carboplatin • Arm 3: pembrolizumab, pemetrexed and carboplatin • Arm 4: volrustomig i.v., taxane (paclitaxel or nab-paclitaxel) and 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 • Data anticipated: 2025

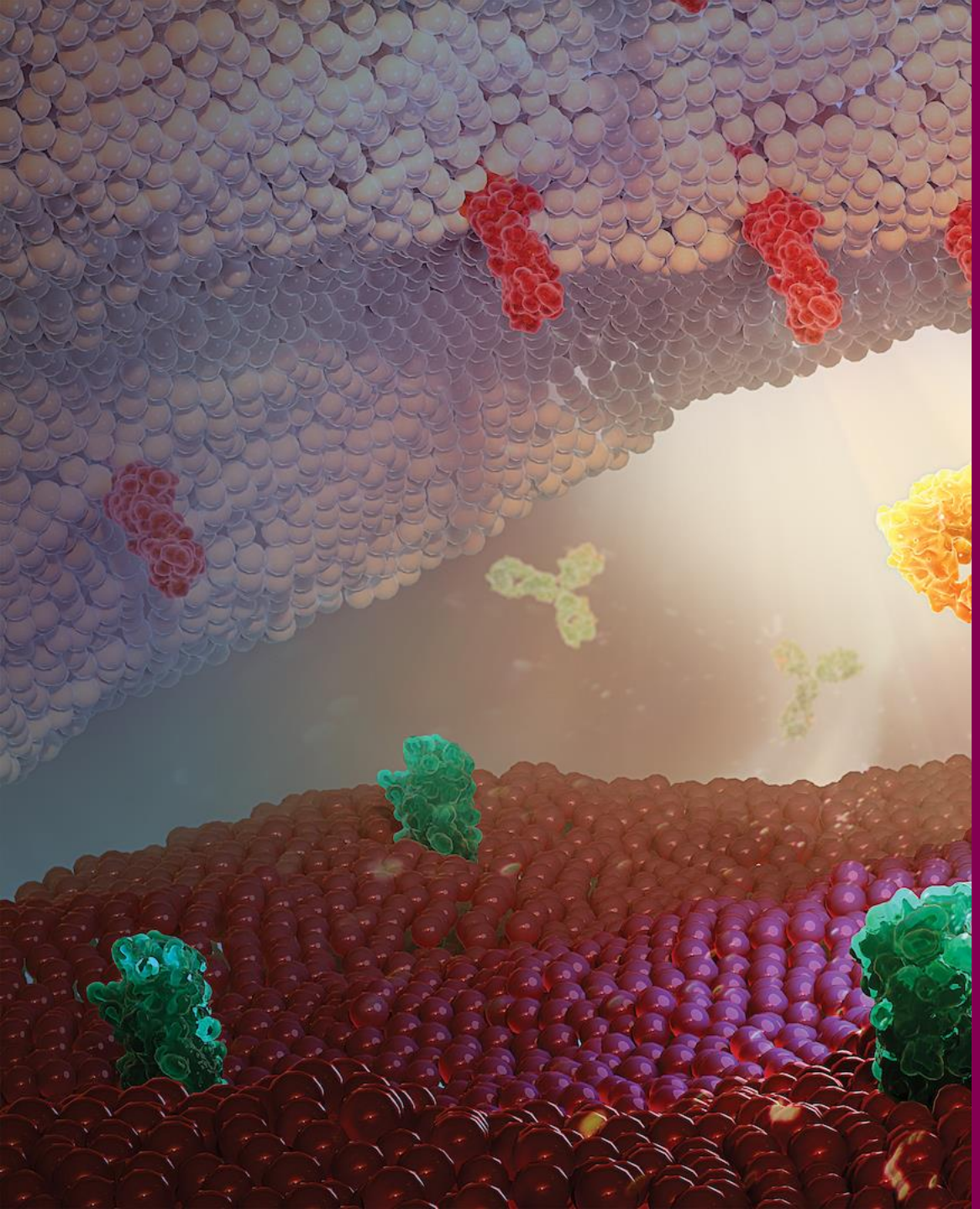
As of 25 April 2024.
Appendix: Glossary.

AZD0901 (CLDN18.2 MMAE ADC)

Solid tumours

Trial	Population	Patients	Design	Endpoints	Status
Phase III **CLARITY –Gastric01** **NCT06346392**	2L+ advanced or metastatic gastric or GEJ adenocarcinoma expressing CLDN18.2		• Open-label, multi-centre, 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 • Global trial	• 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: Q 2024 • Data anticipated: >2025
Phase II **NCT06219941**	Locally advanced unresectable or metastatic solid tumours expressing CLDN18.2	123	• Open-label, multi-centre trial of AZD0901 administered via i.v. • Sub-study 1: AZD0901 monotherapy • Sub-study 2: AZD0901 and anti-cancer agents	• Primary endpoints: AEs, SAEs and ORR • Secondary endpoints: OS, PFS, DoR, DCR, PK parameters and prevalence of ADAs	• FPCD: Q1 2024 • Data anticipated: >2025

As of 25 April 2024.
Appendix: Glossary.



Oncology:
early-stage development

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 study • AZD0120	• Primary endpoints: incidence of AEs, DLTs and ORR • Secondary endpoints: DOR, PFS, OS and PK parameters	• FPCD: Q3 2023 • Data anticipated: >2025

As of 25 April 2024.
Appendix: Glossary.

AZD0171 (anti-LIF mAb)

Cancer

Trial	Population	Patients	Design	Endpoints	Status
Phase II **NCT04999969**	1L metastatic pancreatic ductal adenocarcinoma	126	• Open-label, non-randomised trial • AZD0171 + *Imfinzi* + gemcitabine, nab-paclitaxel	• Primary endpoints: safety, OS at 12 months • Secondary endpoints: ORR, DoR and PFS	• FPCD: Q1 2022 • Data anticipated: H2 2024

As of 25 April 2024.
Appendix: Glossary.

AZD0305 (GPRC5D ADC)

Blood cancers

Trial	Population	Patients	Design	Endpoints	Status
Phase I/II **NCT06106945**	R/R multiple myeloma		• 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: 2025

As of 25 April 2024.
Appendix: Glossary.

AZD0754 (STEAP2 dnTGFbetaRII Armored CAR-T)

Haematologic malignancies

Trial	Population	Patients	Design	Endpoints	Status
Phase I/II **APOLLO** **NCT06267729**	Metastatic prostate cancer	60	• Multi-centre, open label autologous CAR T-cell therapy trial • Arm 1: AZD0754 monotherapy administered via i.v.	• Primary endpoints: safety and tolerability • Secondary endpoints: PSA, BOR, ORR, TTRDoR, DRR, DCR, rPFS, OS, SSRE, Cmax, Tmax, Clast, Tlast, PK parameters, STEAP2 expression and percentage change in tumour size	• FPCD: Q1 2024 • Data anticipated: >2025

As of 25 April 2024.
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	138	• Open-label trial • Arm 1: recurrent GBM, AZD1390 + RT in dose escalation cohorts • 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: 2025

As of 25 April 2024.
Appendix: Glossary.

AZD2068 (FPI-2068, EGFR cMET bispecific mAb)

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	• Data anticipated: >2025 • Initiating

As of 25 April 2024.
Appendix: Glossary.

AZD3470 (PRMT5)

Solid tumours and blood cancer

Trial	Population	Patients	Design	Endpoints	Status
Phase I PRIMROSE NCT06130553	MTAP-deficient advanced solid tumours	210	• Arm 1: AZD3470 • Global trial – 8 countries	• Primary endpoints: safety and tolerability • Secondary endpoints: PK parameters and clinical efficacy	• FPCD: Q1 2024 • Data anticipated: 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: >2025

As of 25 April 2024.
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	150	• Module 1: AZD5335 monotherapy • Module 2: AZD5335 in combination with saruparib	• Primary endpoints: safety and tolerability • Secondary endpoints: efficacy and PK parameters	• FPCD: Q3 2023 • Data anticipated: >2025

As of 25 April 2024.
Appendix: Glossary.

AZD5851 (armoured TGFbetaRIIDN GPC3 CAR-T)

Gastrointestinal cancer

Trial	Population	Patients	Design	Endpoints	Status
Phase I/II ATHENA NCT06084884	GPC3-positive advanced/recurrent HCC	93	• 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), 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: Q1 2024 • Data anticipated: >2025

As of 25 April 2024.
Appendix: Glossary.

AZD5863 (CLDN18.2 x CD3 bispecific antibody)

Solid tumours

Trial	Population	Patients	Design	Endpoints	Status
Phase I **NCT06005493**	Advanced or metastatic solid tumours with CLDN18.2 expression	200	• 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 endpoint (Part A): safety and tolerability • Primary endpoint (Part B): safety, tolerability and preliminary anti-tumour activity • Secondary endpoints: preliminary anti-cancer activity, PK parameters and immunogenicity	• FPCD: Q4 2023 • Data anticipated: >2025

As of 25 April 2024.
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 assessing anti-CLDN18.2 CAR-T cell therapy with 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 anticipated: >2025

As of 25 April 2024.
Appendix: Glossary.

AZD8205 (B7H4 ADC)

Solid tumours

Trial	Population	Patients	Design	Endpoints	Status
Phase I/II NCT05123482	Breast cancer, BTC, ovarian cancer, endometrial cancer	340	• Open-label dose escalation and expansion study of AZD8205 as monotherapy and in combination with anti-cancer agents in participants with advanced solid tumours (BLUESTAR)	• 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: >2025

As of 25 April 2024.
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	• Arm 1: AZD8421 • Arm 2: AZD8421+ camizestrant + abemaciclib • Arm 3: AZD8421 + camizestrant + ribociclib • Arm 4: AZD8421 + camizestrant + palbociclib • Global trial – 4 countries	• Primary endpoints: safety and tolerablility • Secondary endpoints: PK parameters	• FPCD: Q4 2023 • Data anticipated: 2025

As of 25 April 2024.
Appendix: Glossary.

AZD9574 (PARP1-sel BBB inhibitor)

Solid tumours

Trial	Population	Patients	Design	Endpoints	Status
Phase I/IIa **CERTIS-1** **NCT05417594**	Advanced solid malignancies	490	• 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 + datopotamab deruxtecan	• 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: >2025

As of 25 April 2024.
Appendix: Glossary.

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	108	• Escalation phase, open-label, multi-centre trial • Arm 1: AZD9592 • Arm 2: AZD9592 + osimertinib • Arm 3: AZD9592 + 5FU + bevacizumab	• Primary endpoints (escalation): safety and tolerability • Primary endpoints (expansion): safety and tolerability, anti-tumour activity • Secondary endpoints (escalation): PK parameters, immunogenicity, anti-tumour activity • Secondary endpoints (expansion): PK parameters and immunogenicity	• FPCD: Q1 2023 • Data anticipated: 2025

As of 25 April 2024.
Appendix: Glossary.

AZD9829 (CD123 TOP1i ADC)

Blood cancers

Trial	Population	Patients	Design	Endpoints	Status
Phase I/II **NCT06179511**	CD123-positive haematological malignancies	60	• Open-label, multicentre 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	• FPCD: Q1 2024 • Data anticipated: 2025

As of 25 April 2024.
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: Q1 2023

As of 25 April 2024.
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 study 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: >2025

As of 25 April 2024.
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 endpoints (Phase Ia): incidence of AEs defined as DLTs • Primary endpoints (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 • Data anticipated: 2025

As of 25 April 2024.
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 the TP53 R175H mutation	24	• Open-label, single-arm, multi-centre trial with dose escalation	• 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: 2025

As of 25 April 2024.
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: Q1 2023

As of 25 April 2024.
Appendix: Glossary.

sabestomig (AZD7789, PD-1/TIM3 bispecific mAb)

Cancer

Trial	Population	Patients	Design	Endpoints	Status
Phase I/IIa **NCT04931654**	NSCLC, gastric cancer and other tumours	192	• Open-label, non-randomised dose escalation and dose expansion trial • Part A: dose escalation in post-IO NSCLC patients with sabestomig i.v. monotherapy • Part B: dose expansion in post-IO and IO-naïve NSCLC patients and also post-IO gastric patients with sabestomig i.v. monotherapy • Global trial	• Primary endpoints: AE, SAE, DLTs and ORR • Secondary endpoints: ORR, DCR, DoR, PFS, OS, PK parameters, ADA and ctDNA	• FPCD: Q4 2021 • Data anticipated: 2025
Phase I/II **NCT05216835**	R/R classical Hodgkin lymphoma	180	• Cohort A: dose escalation where patients with anti-PD-1/PD-L1 exposed R/R cHL will receive sabestomig • Cohort B1: dose expansion where patients with anti-PD-1/PD-L1 exposed R/R cHL will receive sabestomig once the recommended Phase II dose (RP2D) has been determined • Cohort B2: dose expansion where patients with anti-PD-1/PD-L1 naïve R/R cHL will receive sabestomig once the RP2D has been determined	• Primary endpoints (Cohort A): AE and DLTs • Primary endpoints (Cohort B1): AE and ORR • Primary endpoints (Cohort B2): AE and CRR • Secondary endpoints (Cohort A): CRR, ORR, DoR, DoCR, PFS, OS, ADA and PK parameters • Secondary endpoints (Cohort B1 and B2): DoR, DoCR, PFS, OS, ADA and PK parameters	• FPCD: Q2 2022 • Data anticipated: >2025

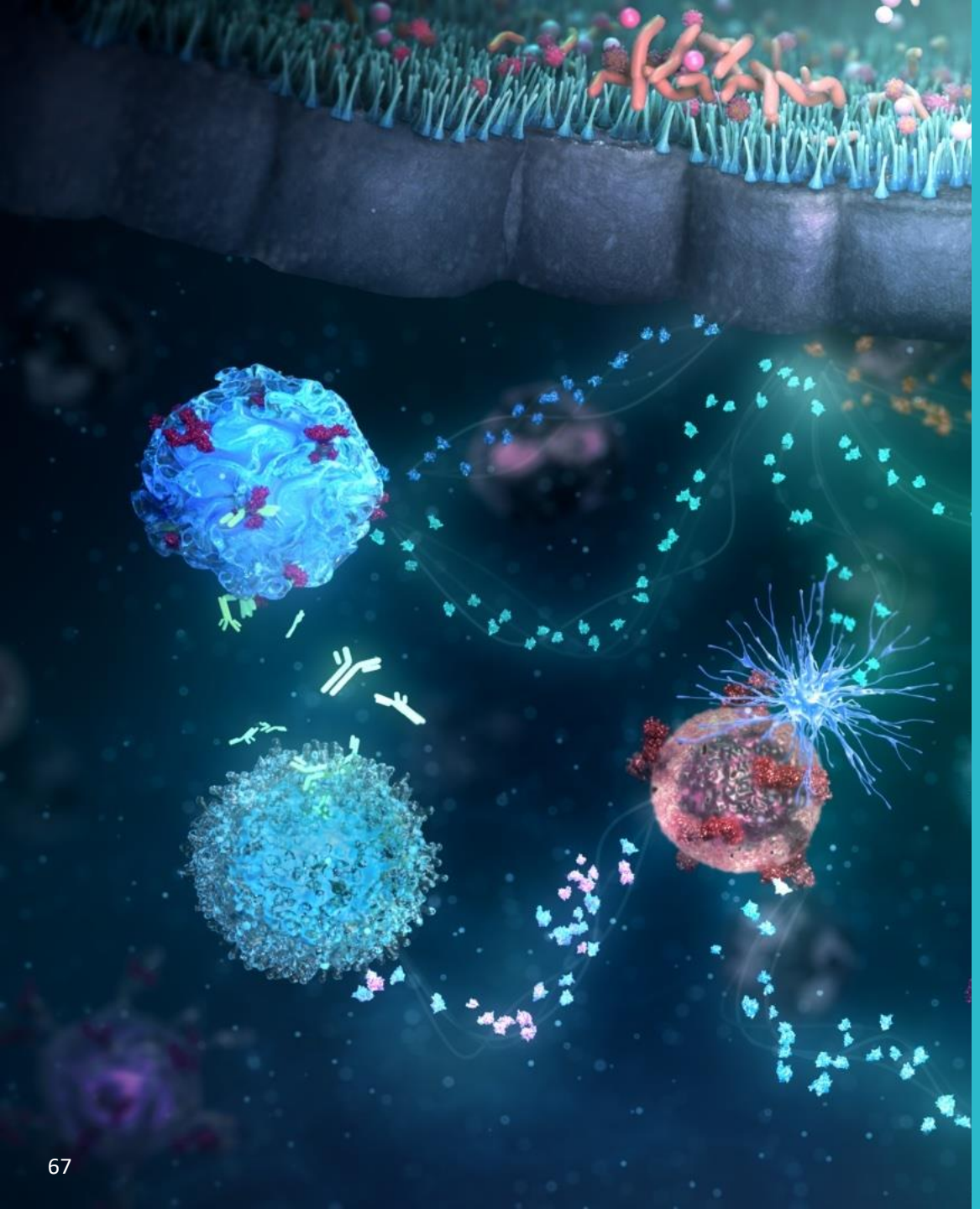
As of 25 April 2024.
Appendix: Glossary.

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

Haematologic malignancies

Trial	Population	Patients	Design	Endpoints	Status
Phase I/II NCT06137118	R/R B-ALL	120	• Multi-centre, open-label, single-arm dose escalation and dose optimisation study	• 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: >2025
Phase I NCT04594642	R/R B-cell non-Hodgkin lymphoma	116	• Multi-centre, open-label, dose escalation and dose expansion trial • AZD0486	• 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: >2025

As of 25 April 2024.
Appendix: Glossary.



BioPharmaceuticals: approved medicines and late-stage development

AstraZeneca

Andexxa (anti-factor Xa reversal)

Haematology

Trial	Population	Patients	Design	Endpoints	Status
Phase IV **I8-513 (post-launch)** **NCT03661528**	Acute intracranial haemorrhage	1200	• Arm 1: *Andexxa* • Arm 2: usual care • Global trial	• Primary endpoint: proportion of patients with good or excellent haemostatic efficacy as rated by an independent adjudication committee • Secondary endpoint: change from baseline in anti-factor Xa activity	• FPCD: Q2 2019 • Data readout: Q2 2023 • Primary endpoint met
Phase II **19-515** **NCT04233073**	Urgent surgery	10	• Arm 1: *Andexxa*	• Primary endpoint: proportion of patients with good or excellent intraoperative haemostatic efficacy as determined by the surgeon's assessment and confirmed by an independent adjudication committee • Secondary endpoint: percent change from baseline in anti-factor Xa activity	• FPCD: Q2 2021 • LPCD: Q1 2022 • Data readout: Q4 2022

As of 25 April 2024.
Appendix: Glossary.

Farxiga (SGLT2 inhibitor)

Heart failure and chronic kidney disease

Trial	Population	Patients	Design	Endpoints	Status
Phase III **DAPA-MI** **NCT04564742**	Myocardial infarction	6400	• Arm 1: Farxiga 10mg QD • Arm 2: placebo • Global trial – 2 countries	• Primary endpoint: time to the first occurrence of any of the components of the composite (hospitalisation for HF or CV death)	• FPCD: Q4 2020 • Data readout: Q4 2023 • Primary endpoint met

As of 25 April 2024.
Appendix: Glossary.

roxadustat (HIF-PH inhibitor)

Anaemia

Trial	Population	Patients	Design	Endpoints	Status
Phase II/III **NCT03303066** **Partnered (FibroGen)**	Anaemia in lower-risk MDS patients	43	• Open-label roxadustat lead-in • Arm 1: roxadustat • Arm 2: placebo • China only	• Primary endpoint: haemoglobin response	• FPCD: Q2 2018 • LPCD: Q1 2023 • Data readout: Q2 2023 • Primary endpoint met

As of 25 April 2024.
Appendix: Glossary.

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	• FPCD: Q1 2020 • Data anticipated: 2025+
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

As of 25 April 2024.
Appendix: Glossary.

balcinrenone/dapagliflozin (MR modulator + SGLT2 inhibitor)

Heart failure

Trial	Population	Patients	Design	Endpoints	Status
Phase III **BalanceD-HF** **NCT06307652**	Heart failure 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 hospitalization; 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-randomization; and time do death from any cause	• FPCD: Q2 2024 • Data anticipated: >2025

As of 25 April 2024.
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 medications including patients with resistant hypertension	720	• Arm 1: baxdrostat 1mg QD • Arm 2: baxdrostat 2mg QD • Arm 3: placebo QD • Global trial – 29 countries	• 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 • Data anticipated: 2025
Phase III **Bax24** **NCT06168409**	Resistant hypertension on three or more medications	212	• Arm 1: baxdrostat 2mg QD • Arm 2: placebo QD • Global trial – 29 countries	• Primary endpoint: effect of baxdrostat vs. placebo on ambulatory 24-hour average systolic blood pressure at Week 12	• Initiating • Data anticipated: 2025
Phase III **BaxAsia** **NCT06344104**	Uncontrolled hypertension on two or more medications including patients with resistant hypertension	300	• Arm 1: baxdrostat 1mg QD • Arm 2 baxdrostat 2mg QD • Arm 3: placebo QD • Global Trial – 10 countries	• 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 and safety and tolerability	• Initiating • Data anticipated: >2025

As of 25 April 2024.
Appendix: Glossary.

baxdrostat (selective aldosterone synthase inhibitor)

Hypertension

Trial	Population	Patients	Design	Endpoints	Status
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 • Data anticipated: H2 2024
Phase II **HALO-OLE** **NCT05459688**	Patients with uncontrolled hypertension who have completed study CIN-107-124	175	• Arm 1: baxdrostat 2mg QD • US only	• Primary endpoints: safety and tolerability	• FPCD: Q2 2022 • Data readout: Q1 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 • Data anticipated: H2 2024
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 • Data anticipated: H2 2024
Phase I **NCT06357520**	Healthy volunteers	14	• Arm 1: baxdrostat 2mg and itraconazole 200mg • US only	• Primary endpoint: AUCinf and Cmax	• Initiating • Data anticipated: H2 2024

As of 25 April 2024.
Appendix: Glossary.

baxdrostat/dapagliflozin

CKD

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: >2025

As of 25 April 2024.
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	1500	• Randomised, parallel, multi-centre, double-blind trial • Arm 1: zibotentan/dapagliflozin dose A or dose B • Arm 2: dapagliflozin • Global trial	• 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 • Data anticipated: >2025
Phase IIb **ZENITH-CKD** **NCT04724837**	CKD	447	• Arm 1: zibotentan dose A + dapagliflozin 10mg QD • Arm 2: zibotentan dose B + dapagliflozin 10mg QD • Arm 3: dapagliflozin 10mg + placebo QD • Global trial	• Primary endpoint: change in log-transformed UACR from baseline to Week 12 zibotentan dose B/dapagliflozin 10mg vs. dapagliflozin 10mg • Secondary endpoints: change in log-transformed UACR from baseline to Week 12 zibotentan dose A/dapagliflozin 10mg vs. dapagliflozin 10mg; change in blood pressure, least squares mean change of UACR, change in eGFR at predetermined timepoints and number of participants experiencing adverse events	• FPCD: Q2 2021 • Data readout: Q3 2023 • Primary endpoint met

As of 25 April 2024.
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 • Global trial	• 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 anticipated: 2025

As of 25 April 2024.
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	1910	• 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: time to first severe asthma exacerbation	• FPCD: Q3 2022 • Data anticipated: 2025
Phase III MANDALA NCT03769090 Managed by Avillion (Avillion)	Moderate to severe asthma	3132	• Randomised, double-blind, multi-centre, parallel group • Treatments: minimum 24-week treatment period • Arm 1: *Airsupra* (budesonide albuterol) MDI 80/180µg prn • Arm 2: *Airsupra* MDI 160/180µg prn • Arm 3: AS MDI 180µg prn • Global trial	• Primary endpoint: time to first severe asthma exacerbation • Secondary endpoints: severe exacerbation rate (annualised); total corticosteroid exposure over the treatment period; Asthma Control Questionnaire -5 change from baseline and responder analysis at Week 24; Asthma Quality of Life questionnaire for 12 years and older/Paediatric Asthma Quality of Life questionnaire change from baseline and responder analysis at Week 24	• FPCD: Q4 2018 • LPCD: Q1 2021 • Data readout: Q3 2021 • Primary endpoint met
Phase III DENALI NCT03847896 Managed by Avillion (Avillion)	Mild to moderate asthma	1001	• Randomised, double-blind, multi-centre and parallel-group • Treatments: 12-week treatment period • Arm 1: *Airsupra* MDI 80/180µg QID • Arm 2: *Airsupra* MDI 160/180µg QID • Arm 3: *Airsupra* MDI 160µg QID • Arm 4: AS MDI 180µg QID • Arm 5: placebo MDI QID • Global trial	• Dual primary endpoints: change from baseline in FEV1 AUC0-6 hours over 12 weeks; change from baseline in trough FEV1 at Week 12	• FPCD: Q2 2019 • LPCD: Q2 2021 • Data readout: Q3 2021 • Dual primary endpoints met
Phase I NCT06139991	Healthy volunteers	66	• Randomised, double-blind, single-dose, single-center, partial-replicate, 3-way crossover trial • BDA MDI HFO 80/90µg (single dose of 2 inhalations) • BDA MDI HFA 80/90µg (single dose of 2 inhalations)	• Primary endpoints: AUClast, Cmax	• FPCD: Q4 2023 • Data anticipated: H2 2024

As of 25 April 2024.
Appendix: Glossary.

Breztri, *Trixeo* (LAMA/LABA/ICS)

Asthma

Trial	Population	Patients	Design	Endpoints	Status
Phase III **KALOS** **NCT04609878**	Severe asthma	2200	• Randomised, double-blind, double-dummy, parallel group and multi-centre trial • Treatments (24- to 52-week variable length) • Arm 1: *Breztri* 320/28.8/9.6µg BID MDI • Arm 2: *Breztri* 320/14.4/9.6µg BID MDI • Arm 3: *Symbicort* 320/9.6µg BID MDI • Arm 4: *Symbicort* 320/9µg BID pMDI • Global trial	• 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 anticipated: 2025
Phase III **LOGOS** **NCT04609904**	Severe asthma	2200	• Randomised, double-blind, double dummy, parallel group and multi-centre trial • Treatments (24- to 52-week variable length) • Arm 1: *Breztri* 320/28.8/9.6µg BID MDI • Arm 2: *Breztri* 320/14.4/9.6µg BID MDI • Arm 3: *Symbicort* 320/9.6µg BID MDI • Arm 4: *Symbicort* 320/9µg BID pMDI • Global trial	• 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 anticipated: 2025
Phase III **VATHOS** **NCT05202262**	Moderate asthma	630	• Randomised, double-blind, parallel group, multi-centre trial • Treatments (24-week) • Arm 1: *Symbicort* 320/9.6µg BID MDI • Arm 1: *Symbicort* 160/9.6µg BID MDI • Arm 3: BD 320µg BID MDI • Arm 4: open-label *Symbicort* TBH 320/9µg BID • Global trial	• Primary endpoint: change from baseline in FEV1 AUC0-3 at Week 24	• FPCD: Q1 2022 • Data anticipated: 2025
Phase III **LITHOS** **NCT05755906**	Mild to moderate asthma	340	• Randomised, double-blind, parallel group and multi-centre • Treatments (12-week) • Arm 1: *Symbicort* 160/9.6µg BID MDI • Arm 2: BD 160µg BID MDI • Global trial	• 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 anticipated: 2025

As of 25 April 2024.
Appendix: Glossary.

Breztri, *Trixeo* (LAMA/LABA/ICS)

COPD

Trial	Population	Patients	Design	Endpoints	Status
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 HFO 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 anticipated: H2 2024
Phase III **ATHLOS** **NCT06067828**	COPD	180	• Randomised, double-blind, three-treatment, three-period, cross-over trial • Treatments (2-week treatment periods, 2-week washout between treatments) • Arm 1: *Breztri* 320/14.4/9.6µg BID MDI • Arm 2: *Symbicort* 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: 2025
Phase III **THARROS** **NCT06283966**	COPD	5000	• Randomised, double blind, parallel group, multi-centre event-driven trial • comparing BGF MDI 320/14.4/9.6 µg BID with GFF MDI 14.4/9.6 µg 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: >2025

As of 25 April 2024.
Appendix: Glossary.

Fasenra (IL-5R mAb)

Nasal polyposis and other eosinophilic diseases

Trial	Population	Patients	Design	Endpoints	Status
Phase III OSTRO NCT03401229	Patients with severe bilateral nasal polyps who are still symptomatic despite SoC therapy; age 18 to 75 years	413	• Arm 1: *Fasenra* 30mg Q8W s.c. • Arm 2: placebo s.c. • 56-week trial • Global trial – 8 countries	• Primary endpoint: effect of Fasenra on nasal polyp burden and on patient reported nasal blockage	• FPCD: Q1 2018 • LPCD: Q2 2019 • Data readout: Q3 2020 • Co-primary endpoints met
Phase III ORCHID NCT04157335	Patients with eosinophilic chronic rhinosinusitis with severe nasal polyposis; age 18 to 75 years	276	• Arm 1: *Fasenra* 30mg Q8W s.c. • Arm 2: placebo Q8W s.c. • 56-week trial • Global trial – 17 countries	• Primary endpoints: change in endoscopic total nasal polyp score and change in mean nasal blockage score	• FPCD: Q4 2019 • Data anticipated: H2 2024
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 • Global trial – 9 countries	• Primary endpoint: proportion of patients achieving remission (BVAS=0 and OCS dose ≤4mg/day) at Week 36 and Week 48	• FPCD: Q4 2019 • 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	120	• Arm 1: *Fasenra* 30mg Q4W s.c. • Arm 2: placebo Q4W s.c. • 24-week trial with a minimum 1-year open label extension • Global trial – 15 to 18 countries	• Primary endpoint: time to first HES worsening/flare	• FPCD: Q3 2020 • Data anticipated: H1 2024

As of 25 April 2024.
Appendix: Glossary.

Fasenra (IL-5R mAb)

Severe, uncontrolled asthma and COPD

Trial	Population	Patients	Design	Endpoints	Status
Phase III MIRACLE NCT03186209	Severe, uncontrolled asthma despite background controller medication, MD and HD ICS + LABA ± chronic OCS; age 12 to 75 years	695	• Arm 1: *Fasenra* 30mg Q8W s.c. • Arm 2: placebo s.c. • 56-week trial	• Primary endpoint: annual asthma exacerbation rate • Secondary endpoints: pulmonary function, asthma symptoms and other asthma control metrics	• FPCD: Q4 2017 • LPCD: Q4 2021 • Data readout: Q1 2023 • Primary endpoint met
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	642	• Double-blind, placebo-controlled trial • Arm 1: *Fasenra* 100mg Q8W s.c. • Arm 2: placebo Q8W s.c. • 56-week treatment • Global trial – 30 countries	• Primary endpoint: annualised rate of moderate or severe exacerbations over 56 weeks	• FPCD: Q4 2019 • Data anticipated: 2025

As of 25 April 2024.
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. • Global trial	• Primary endpoint: BICLA at Week 52	• FPCD: Q3 2021 • Data anticipated: 2025
Phase III **AZALEA-SLE** **NCT04931563** **Partnered (BMS)**	Moderate to severe SLE	260	• Arm 1: 300mg *Saphnelo* i.v. Q4W • Arm 2: placebo i.v. Q4W • Asia only	• Primary endpoint: BICLA at Week 52	• FPCD: Q4 2021 • Data anticipated: 2025
Phase III **IRIS** **NCT05138133** **Partnered (BMS)**	Active, proliferative LN	360	• Arm 1: *Saphnelo* i.v. • Arm 2: placebo i.v. • Global trial	• Primary endpoint: CRR at Week 52	• FPCD: Q2 2022 • Data anticipated: >2025

As of 25 April 2024.
Appendix: Glossary.

Saphnelo (type I interferon receptor mAb)

Sclerosis

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. • Global trial	• Primary endpoint: CRISS-25 at Week 52	• FPCD: Q4 2023 • Data anticipated: >2025

As of 25 April 2024.
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 • Global trial – 10 countries	• Co-primary endpoint: nasal polyp score and participant reported nasal congestion	• FPCD: Q2 2021 • LPCD: Q4 2023 • Data anticipated: H2 2024
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 • Global trial – 20+ countries	• 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 • Data anticipated: >2025
Phase IIa **COURSE** **NCT04039113** **Partnered (AMGEN)**	Moderate to very severe COPD; age 40 to 80	338	• Arm 1: *Tezspire* s.c. • Arm 2: placebo s.c. • 52-week trial • Global trial – 10 countries	• Primary endpoint: rate of moderate or severe COPD exacerbations	• FPCD: Q3 2019 • LPCD: Q4 2022 • Data anticipated: H1 2024

As of 25 April 2024.
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 • Global trial – 18 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: 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 anticipated: H2 2024

As of 25 April 2024.
Appendix: Glossary.

HFO1234ze (next-generation propellant)

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: HFO 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 anticipated: H2 2024
Phase III NCT05850494	Well-controlled or partially-controlled asthma	52	• Randomised, mulit-centre double-blind, single-dose crossover trial • Arm 1: HFO 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 anticipated: H1 2024
Phase III NCT06075095	COPD	240	• Randomised, placebo-controlled, double-blind, multi-centre, 4-week, 3-way crossover pharmacodynamic trial to assess the equivalence of *Breztri* delivered by pMDI HFO vs. with *Breztri* delivered by MDI HFA • Arm 1: *Breztri* pMDI HFO 320/14.4/9.6µg • Arm 2: *Breztri* pMDI HFA 320/14.4/9.6 µg • Placebo: MDI HFA	• Primary endpoint: changes in FEV1 AUC (0-4) and change in morning pre-dose trough FEV1 • Secondary endpoints: safety and efficacy	• FPCD: Q1 2024 • Data anticipated: 2025

As of 25 April 2024.
Appendix: Glossary.

HFO1234ze (next-generation propellant)

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 HFO 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 endpoint: AUCinf, AUClast and Cmax	• FPCD: Q3 2022 • Data readout: Q4 2023
Phase I NCT05569421	Healthy volunteers	108	• Randomised, double-blind, single-dose, single-centre, partial-replicate, 3-way crossover trial • Arm 1: *Breztri* pMDI HFO 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 endpoint: AUCinf, AUClast and Cmax	• FPCD: Q4 2022 • Data anticipated: H1 2024
Phase I NCT06139991	Healthy volunteers	66	• Randomised, double-blind, single-dose, cross-over trial to assess the equivalence of *Airsupra* delivered by pMDI HFO vs. with *Airsupra* delivered by pMDI HFA • Arm 1: *Airsupra* pMDI HFO 80/90µg (single dose of 2 inhalations) • Arm B: *Airsupra* pMDI HFA 80/90µg (single dose of 2 inhalations)	• Primary endpoint: AUClast and Cmax	• FPCD: Q4 2023 • Data anticipated: H2 2024

As of 25 April 2024.
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	2902	• Randomised, double-blind, placebo-controlled, parallel group • Arm 1: tozorakimab dose i.v. + SoC • Arm 2: placebo i.v. + SoC • Global trial – 38 countries	• 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: >2025
Phase II **FRONTIER-3** **NCT04570657**	Adults with uncontrolled moderate to severe asthma	250	• Randomised, double-blind, placebo-controlled trial • Arm 1: tozorakimab dose 1 s.c. • Arm 2: tozorakimab dose 2 s.c. • Arm 3: placebo s.c. • Global trial – US, Argentina, Germany, Hungary, Poland, South Africa and UK	• Primary endpoint: change from baseline at Week 16 in FEV1 • Secondary endpoints: safety and other efficacy measures	• FPCD: Q4 2020 • LPCD: Q3 2022 • Data readout: Q2 2023

As of 25 April 2024.
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	1060	• 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 • Global trial – 20 countries	• 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: >2025
Phase III TITANIA NCT05158387	Adults with symptomatic COPD with a history of exacerbations	1060	• 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 • Global trial – 19 countries	• 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: >2025
Phase III PROSPERO NCT05742802	Subjects who completed either OBERON or TITANIA will be offered the opportunity to consent (adults with symtomatic COPD with a history of exacerbations)	1596	• Randomised, double-blind, placebo-contolled, paralell-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 • Global trial – 38 countries	• Primary endpoint: time to first severe COPD exacerbation in primary population of former smokers over the treatment period incorporating both the predecessor studies and PROSPERO • Secondary endpoint: time to first severe COPD exacerbation in the overall population of current and former smokers	• FPCD: Q1 2023 • Data anticipated: >2025

As of 25 April 2024.
Appendix: Glossary.

tozorakimab (IL-33 ligand mAb)

COPD

Trial	Population	Patients	Design	Endpoints	Status
Phase III MIRANDA NCT06040086	Adults with symptomatic COPD with a history of exacerbations	1240	• Randomised, double-blind, placebo-controlled, parallel group • Arm 1: tozorakimab dose s.c. + SoC • Arm 2: placebo s.c. + SoC • Global trial – 29 countries	• 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 and former or current smokers) and change in pre-BD FEV1, E-RS:COPD and SGRQ	• FPCD: Q4 2023 • Data anticipated: >2025
Phase II FRONTIER-4 NCT04631016	Adults with COPD and chronic bronchitis	137	• Randomised, double-blind, placebo-controlled, parallel-group, PoC trial • Arm 1: tozorakimab s.c. • Arm 2: placebo s.c. • Global trial – 15 countries	• Primary endpoint: change from baseline at Week 12 in FEV1 • Secondary endpoints: safety and other efficacy measures	• FPCD: Q1 2021 • Data readout: Q3 2023

As of 25 April 2024.
Appendix: Glossary.

Beyfortus (nirsevimab, RSV mAb-YTE)

Infection

Trial	Population	Patients	Design	Endpoints	Status
Phase III MELODY NCT03979313	Healthy infants (born 35 weeks 0 days or greater gestational age)	3012	• Randomised, double-blind, placebo-controlled • Arm 1: *Beyfortus* i.m. • Arm 2: placebo i.m. • Global trial – 31 countries	• Primary endpoint: efficacy • Secondary endpoints: safety, PK parameters and ADA	• Data readout: Q3 2022 • FPCD: Q2 2021 (safety cohort) • LPCD: Q4 2021 (safety cohort) • Data readout: Q3 2022 (safety cohort) • Primary endpoint met • FPCD: Q3 2019 (efficacy cohort) • LPCD: Q1 2020 (efficacy cohort) • Data readout: Q2 2021 (efficacy cohort) • Primary endpoint met
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 endpoints efficacy • Secondary endpoints: safety, PK parameters and ADA	• FPCD: Q4 2021 • Data anticipated: 2025
Phase II/III MEDLEY NCT03959488	High-risk pre-term (born 35 weeks 0 day or less gestational-age) CHD and CLD infants eligible to receive Synagis	925	• Randomised, double-blind, palivizumab-controlled • Arm 1: *Beyfortus* i.m. • Arm 2: *Synagis* i.m. • Global trial – 32 countries	• Primary endpoints: safety and tolerability • Secondary endpoints: PK parameters, ADA and descriptive efficacy	• FPCD: Q3 2019 • LPCD: Q4 2020 • Data readout: Q2 2021 • Safety objective met
Phase II MUSIC NCT04484935	Immunocompromised children who are ≤24 months of age at the time of dose administration	100	• Open-label, uncontrolled, single-dose trial • *Beyfortus* i.m. • Route of administration: i.m. • Global trial – 8 countries	• Primary endpoints: safety and tolerability • Secondary endpoints: PK parameters, ADA and efficacy	• FPCD: Q3 2020 • LPCD: Q1 2022 • Data readout: Q2 2023 • Primary endpoint met

As of 25 April 2024.
Appendix: Glossary.

Evusheld (AZD7442, tixagevimab + cilgavimab)

COVID-19

Trial	Population	Patients	Design	Endpoints	Status
Phase II ENDURE NCT05375760	Adults and pediatric individuals (≥12 years of age weighing at least 40kg) who are moderate to severely immunocompromised due to an underlying disease or are taking immunosuppressive medications and therefore unable to mount an adequate immune response	251	• Randomised, open-label, dose-ranging to assess safety, immunogenicity, PK and PD profiles in pre-exposure prophylaxis • Arm 1: *Evusheld*, dose regimen 1 • Arm 2: *Evusheld*, dose regimen 2 • US only	• Primary endpoints: safety and tolerability, incidence of ADA • Secondary endpoints: individual serum concentration; GMTs and GMFR in severe acute respiratory CoV-2 neutralizing antibodies	• FPCD: Q2 2022 • LPCD: Q3 2022 • Data readout: Q1 2024 • Primary endpoint met
Phase I NCT05166421	Healthy adults; age ≥18 years	207	• Open-label, randomised, three-arm, single-dose trial • Arm 1: *Evusheld* administered as a single co-formulated dose (clonal cell line material) • Arm 2: *Evusheld* administered as two separate doses (clonal cell line material) • Arm 3: *Evusheld* administered as two separate doses (cell pool material) • *Evusheld* (1:1:1) • US only	• Primary endpoints: safety and PK parameters	• FPCD: Q1 2022 • LPCD: Q3 2022 • Data readout: Q4 2023 • Primary endpoint met
Phase I TRUST NCT05281601 (No partner)	Pediatric participants ≥29 weeks gestational age to <18 years at increased risk of developing severe SARS-CoV-2 infection	100	• Open-label, single-dose, three cohort trial • Cohort 1: pre-exposure prophylaxis • Cohort 2: mild-to-moderate COVID-19 • Cohort 3: severe COVID-19 • *Evusheld* • US only	• Primary endpoints: safety, tolerability and PK parameters	• FPCD: Q1 2022 • Data anticipated: H2 2024

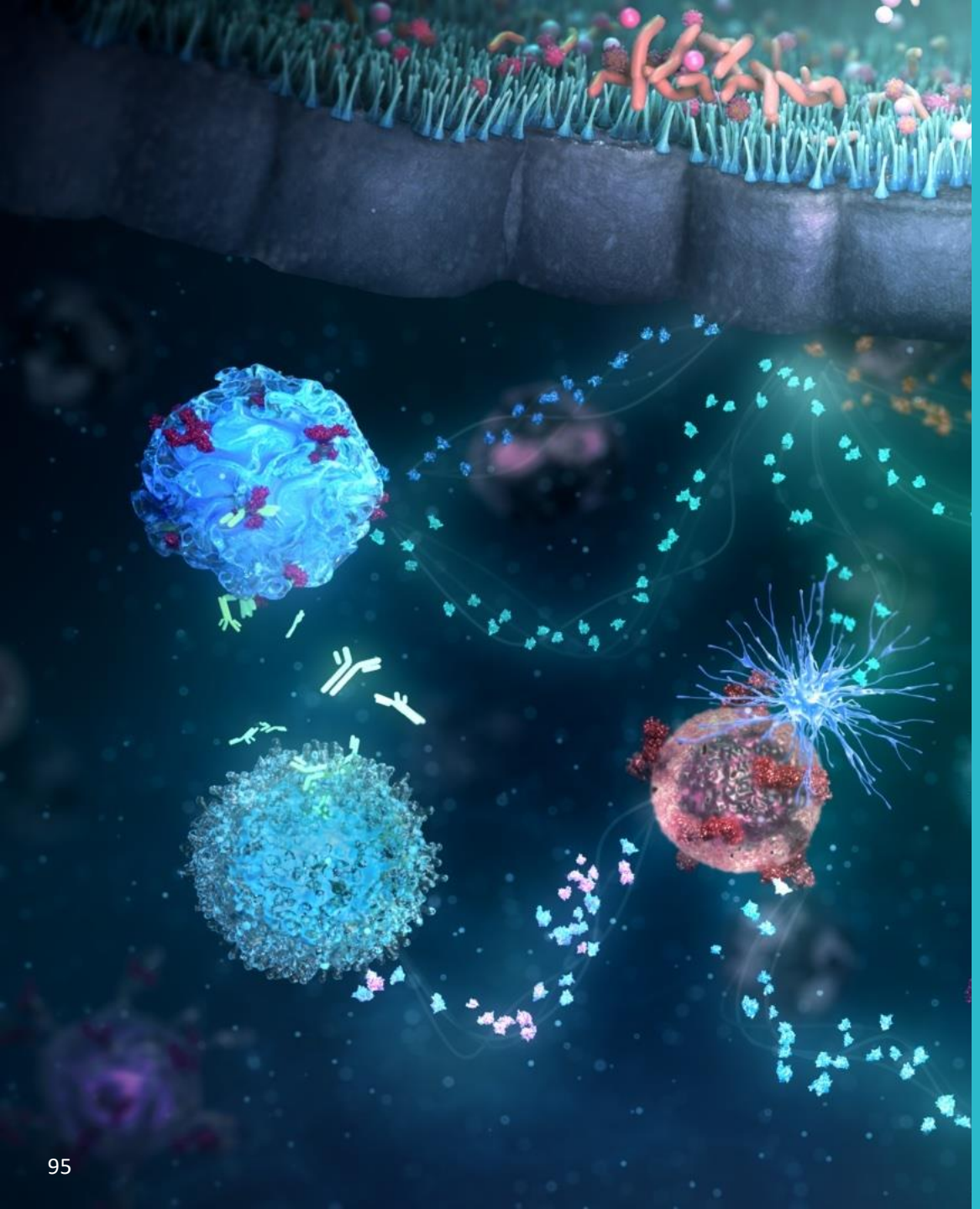
As of 25 April 2024.
Appendix: Glossary.

sipavibart (AZD3152, 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* • Global trial	• 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 anticipated: H1 2024
Phase I **LITTLE DIPPER** **NCT05872958**	Healthy adult participants; age 18 to 55 years	96	• Phase I, double-blind, placebo-controlled, multi-centre, dose exploration study • to evaluate the safety and PK of AZD3152 in healthy adult participants across different dose levels and routes of administration • Approximately 96 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 25 April 2024.
Appendix: Glossary.



BioPharmaceuticals: early-stage development

AstraZeneca

AZD0780 (PCSK9 inhibitor)

Dyslipidaemia

Trial	Population	Patients	Design	Endpoints	Status
Phase II PURSUIT NCT06173570	Dyslipidaemia	175	• Randomised trial with equal distribution across 5 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 • Data anticipated: 2025
Phase I NCT05384262	Healthy adults	132	• Randomised, placebo-controlled SAD/MAD trial	• Primary endpoints: safety and tolerability	• FPCD: Q2 2022 • Data anticipated: H2 2024
Phase I NCT05787002	Healthy volunteers	16	• Open-label, two-period, two-sequence crossover study to assess the effect of AZD0780 on the PK of Crestor	• Primary endpoints: PK parameters, safety and tolerability	• FPCD: Q1 2023 • LPCD: Q2 2023 • Data readout: Q4 2023
Phase I NCT05817461	Healthy volunteers	8	• Open-label, two-part sequential human ADME study	• Primary endpoints: mass balance recovery, absorption, metabolism, excretion of [14C]AZD0780 and absolute bioavailability of AZD0780 • Secondary endpoints: safety and tolerability	• FPCD: Q2 2023 • LPCD: Q2 2023 • Data readout: Q4 2023

As of 25 April 2024.
Appendix: Glossary.

AZD1705 (lipid lowering)

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 • Data anticipated: 2025

As of 25 April 2024.
Appendix: Glossary.

AZD2373

Chronic kidney disease

Trial	Population	Patients	Design	Endpoints	Status
Phase I **NCT04269031**	Healthy volunteers	30	• SAD dose escalation in 6 cohorts with 6 volunteers receiving AZD2373 and 2 volunteers receiving placebo in each cohort • Arm 1: AZD2373 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 AZD2373 and 2 volunteers per cohort receiving placebo • Arm 1: AZD2373 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 25 April 2024.
Appendix: Glossary.

AZD2389 (anti-fibrotic mechanism)

MASH

Trial	Population	Patients	Design	Endpoints	Status
Phase I NCT06138795	Healthy volunteers	104	• Randomised, placebo-controlled SAD/MAD trial	• Primary endpoints: safety and tolerability	• FPCD: Q4 2023 • Data anticipated: H2 2024

As of 25 April 2024.
Appendix: Glossary.

AZD2693 (antisense oligonucleotide)

MASH

Trial	Population	Patients	Design	Endpoints	Status
Phase IIb **FORTUNA** **NCT05809934**	NASH 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. • Global trial	• Primary endpoints: efficacy, safety and tolerability of AZD2693	• FPCD: Q2 2023 • Data anticipated: >2025
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 anticipated: H1 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 study • US only	• Primary endpoints: safety, tolerability and PK parameters	• FPCD: Q3 2023 • Data anticipated: H2 2024

As of 25 April 2024.
Appendix: Glossary.

AZD3427 (relaxin)

Heart failure

Trial	Population	Patients	Design	Endpoints	Status
Phase II **Re-PHiRE** **NCT05737940**	Heart failure and pulmonary hypertension due to left heart disease	220	• 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 • Global trial – US, Canada, China, Japan, Czech Republic, Italy, Spain, Netherlands, Poland, UK, Austria, Germany, Denmark and Sweden	• Primary endpoint: change in PVR from baseline to Week 25 vs. placebo as measured by right heart catheterisation	• FPCD: Q2 2023 • Data anticipated: 2025

As of 25 April 2024.
Appendix: Glossary.

AZD4144 (inflammation modulator)

Cardiorenal disease

Trial	Population	Patients	Design	Endpoints	Status
Phase I **NCT06122714**	Healthy volunteers	104	• Randomised, single-blind, placebo-controlled, single ascending dose SAD/MAD sequential group trial	• Primary endpoints: safety and tolerability • Secondary endpoints: PK parameters	• FPCD: Q4 2023 • Data anticipated: H2 2024

As of 25 April 2024.
Appendix: Glossary.

AZD5462 (relaxin)

Heart failure

Trial	Population	Patients	Design	Endpoints	Status
Phase I **NCT04994106**	Healthy volunteers	98	• Single-centre SAD and MAD • Part A: SAD (8 cohorts) • Arm 1: AZD5462 • Arm 2: placebo • Part B: MAD (5 cohorts) • Arm 1: AZD5462 • Arm 2: placebo • US only	• Primary endpoints: safety and tolerability	• FPCD: Q4 2021 • LPCD: Q3 2022 • Data readout: Q2 2023

As of 25 April 2024.
Appendix: Glossary.

AZD6234 (long-acting amylin)

Obesity with related comorbidities

Trial	Population	Patients	Design	Endpoints	Status
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		• Randomised, single-blind, placebo-controlled trial with repeated doses of AZD6234 or placebo via s.c. injection	• Primary endpoint: safety and terability of repeat doses	• FPCD: Q4 2023 • Data anticipated: H2 2024

As of 25 April 2024.
Appendix: Glossary.

AZD7503 (antisense oligonucleotide)

MASH

Trial	Population	Patients	Design	Endpoints	Status
Phase I NCT05143905	Healthy volunteers	56	• SAD, 7 cohorts • Arm 1: AZD7503 s.c. • Arm 2: placebo s.c. • US only	• Primary endpoints: safety and tolerability • Secondary endpoint: PK	• FPCD: Q4 2021 • Data readout: Q4 2023 • Trial discontinued due to strategic portfolio prioritisation
Phase I NCT05560607	NAFLD or NASH	14	• Single-centre, open-label Phase I study to assess knockdown of hepatic HSD17B13 mRNA PK, safety and tolerability following multiple doses of AZD7503	• Primary endpoint: safety and tolerability • Secondary endpoint: change in HSD17B13 mRNA expression	• FPCD: Q3 2022 • Data anticipated: H2 2024 • Trial discontinued due to strategic portfolio prioritisation
Phase I NCT05864391	NASH F1-F3	60	• Randomised, single-blind, MAD trial	• Primary endpoint: safety and tolerability • Secondary endpoint: PK parameters	• FPCD: Q3 2023 • Data anticipated: H2 2024 • Trial discontinued due to strategic portfolio prioritisation

As of 25 April 2024.
Appendix: Glossary.

AZD9550 (GLP-1-glucagon agonist)

MASH

Trial	Population	Patients	Design	Endpoints	Status
Phase I NCT05848440	Healthy volunteers	64	• SAD trial	• Primary endpoint: safety and tolerability	• FPCD: Q2 2023 • LPCD: Q4 2023 • Data anticipated: H1 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 and tolerability and PK parameters	• FPCD: Q4 2023 • Data anticipated: 2025

 As of 25 April 2024.
Appendix: Glossary.

balcinrenone/dapagliflozin (MR modulator + SGLT2 inhibitor)

Heart failure

Trial	Population	Patients	Design	Endpoints	Status
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 • Global trial – 19 countries	• 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

As of 25 April 2024.
Appendix: Glossary.

MEDI6570

Cardiovascular disease

Trial	Population	Patients	Design	Endpoints	Status
Phase IIb GOLDILOX NCT04610892	Post-myocardial infarction	400	• Evaluation of anti-inflammatory potential and effect on surrogates for atherosclerotic and heart failure events • Arm 1: MEDI6570 (high dose) • Arm 2: MEDI6570 (medium dose) • Arm 3: MEDI6570 (low dose) • Arm 4: placebo • Global trial – US, Canada, Hungary, Japan, Czech Republic, Italy, Spain, Netherlands, Poland, UK, Australia and Russia	• Primary endpoints: safety and efficacy	• FPCD: Q4 2020 • LPCD: Q4 2022 • Trial discontinued due to strategic portfolio prioritisation

As of 25 April 2024.
Appendix: Glossary.

mitiperstat (MPO inhibitor)

Cardiovascular disease

Trial	Population	Patients	Design	Endpoints	Status
Phase IIb ENDEAVOR NCT04986202	HFpEF	711	• Randomised, double-blind • Arm 1: 2.5mg mitiperstat • Arm 2: 5mg mitiperstat • Arm 3: placebo • Global trial	• Primary endpoints: safety and efficacy	• FPCD: Q3 2021 • Data anticipated: H1 2024
Phase I NCT05236543	Healthy volunteers	14	• Open-label • mitiperstat vs. mitiperstat and itraconazole • UK only	• Primary endpoints: PK parameters • Secondary endpoints: safety and tolerability	• FPCD: Q1 2022 • LPCD: Q3 2022 • Data readout: Q1 2023
Phase I NCT05457270	Healthy volunteers	30	• Open-label • 2-period, 2-treatment, single-dose, crossover trial • Period 1: single oral dose mitiperstat Formulation A or B on Day 1 • Period 2: single oral dose mitiperstat Formulation A or B on Day 1 • US only	• Primary endpoints: relative bioavailability and PK parameters • Secondary endpoints: safety and tolerability	• FPCD: Q3 2022 • LPCD: Q3 2022 • Data readout: Q1 2023

As of 25 April 2024.
Appendix: Glossary.

mitiperstat (MPO inhibitor)

MASH

Trial	Population	Patients	Design	Endpoints	Status
Phase II **COSMOS** **NCT05638737**	NASH	90	• Randomised, placebo-controlled, double-blind • Arm 1: 5mg mitiperstat • Arm 2: placebo • Global trial	• Primary endpoints: safety, tolerability and PD parameters	• FPCD: Q1 2023 • Data anticipated: H1 2024
Phase I **NCT05751759**	Participants with hepatic impairment and participants with normal hepatic function	32	• Phase I, single dose, non-randomised, open-label, parallel-group trial	• Primary endpoints: safety, tolerability and PK parameters	• FPCD: Q1 2023 • Data anticipated: H2 2024

As of 25 April 2024.
Appendix: Glossary.

atuliflapon (FLAP inhibitor)

Asthma

Trial	Population	Patients	Design	Endpoints	Status
Phase IIa **FLASH** **NCT05251259**	Patients with moderate-to-severe uncontrolled asthma	667	• 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: 2025

As of 25 April 2024.
Appendix: Glossary.

AZD0292 (Psl-PcrV N3Y-bispecific mAb)

Bronchiectasis

Trial	Population	Patients	Design	Endpoints	Status
Phase I **NCT06311760**	Healthy volunteers	24	• 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: placebo administered via i.v. infusion	• Primary endpoints: AEs and participants with AESI • Secondary endpoints: Cmax, AUClast, AUCinfinity and ADA	• Initiating • Data anticipated: 2025

As of 25 April 2024.
Appendix: Glossary.

AZD1163 (bispecific antibody)

Rheumatoid arthritis

Trial	Population	Patients	Design	Endpoints	Status
Phase I **NCT06103877**	Healthy volunteers	64	• Randomised, double-blind, placebo-controlled SAD/MAD trial • Part 1 (SAD): 9 cohorts with 8 i.v. administered dose levels and 1 s.c. administered dose level of AZD1163 • Part 2 (MAD): 2 s.c. dose levels of AZD1163	• Primary endpoint: number of participants with AEs • Secondary endpoints: AUCinf, AUClast and Cmax	• FPCD: Q4 2023 • Data anticipated: 2025

As of 25 April 2024.
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	• Multicentre, 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: >2025
Phase I **NCT04769869**	Healthy volunteers and patients with mild asthma	137	• SAD/MAD/POM trial • Part 1 SAD • Arm 1: AZD4604 (DPI) • Arm 2: placebo (DPI) • Part 2 MAD • Arm 1: AZD4604 (DPI) • Arm 2: placebo (DPI) • Part 3 POM • Arm 1: AZD4604 (DPI) • Arm 2: placebo (DPI) • UK only	• Primary endpoints: safety and tolerability • Secondary endpoints: PK parameters and FENO	• FPCD: Q4 2021 • Data readout: Q3 2023

As of 25 April 2024.
Appendix: Glossary.

AZD5055 (oral porcupine inhibitor)

IPF and other ILDs with progressive fibrosis

Trial	Population	Patients	Design	Endpoints	Status
Phase I NCT05134727	Healthy volunteers	90	• SAD/MAD trial • Part 1: SAD • Arm 1: AZD5055 (oral suspension) • Arm 2: placebo (oral suspension) • Part 2: MAD • Arm 1: AZD5055 (oral suspension) • Arm 2: placebo (oral suspension)	• Primary endpoints: safety and tolerability • Secondary endpoints: PK parameters	• FPCD: Q4 2021 • LPCD: Q2 2023 • Trial discontinued due to strategic portfolio prioritisation
Phase I NCT05630677	Healthy volunteers	18	• BA trial to compare film-coated tablet with oral suspension and to assess the effect of food and an acid reducing agent on PK of AZD5055 in healthy volunteers	• Primary endpoints: bioavailability and PK parameters	• FPCD: Q4 2022 • LPCD: Q1 2023 • Trial discontinued due to strategic portfolio prioritisation

As of 25 April 2024.
Appendix: Glossary.

AZD6793 (IRAK4)

Inflammatory diseases

Trial	Population	Patients	Design	Endpoints	Status
Phase I **NCT05662033**	Healthy volunteers	133	• Single-blind, randomised, placebo-controlled study to investigate the safety, tolerability and PK of oral AZD6793 following single and multiple ascending doses in healthy subjects	• Primary endpoints: safety and tolerability • Secondary endpoint: PK parameters	• FPCD: Q4 2022 • Data anticipated: H2 2024

As of 25 April 2024.
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 • Arm 1: AZD6912 • Arm 2: placebo	• Primary endpoint: incidence of AEs • Secondary endpoint: PK parameters	• FPCD: Q4 2023 • Data anticipated: 2025

As of 25 April 2024.
Appendix: Glossary.

AZD7798 (humanised mAb)

Crohn's disease

Trial	Population	Patients	Design	Endpoints	Status
Phase I **NCT05452304**	Japanese and Chinese healthy volunteers	144	• SAD repeating dose trial • Arm 1: AZD7798 • Arm 2: placebo	• Primary endpoints: safety and tolerability • Secondary endpoints: PK parameters and immunogenicity	• FPCD: Q3 2022 • Data readout: Q4 2023

As of 25 April 2024.
Appendix: Glossary.

AZD8630 (inhaled TSLP)

Asthma

Trial	Population	Patients	Design	Endpoints	Status
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

As of 25 April 2024.
Appendix: Glossary.

mitiperstat (MPO inhibitor)

COPD

Trial	Population	Patients	Design	Endpoints	Status
Phase II **CRESCENDO** **NCT05492877**	Moderate to severe COPD; age 40 to 80	406	• Randomised, double-blind trial • Arm 1: 5mg mitiperstat • Arm 2: placebo • Global trial – 14 countries	• Primary endpoint: time to first COPD CompEx event • Secondary endpoints: plasma concentration-time profiles, PK parameters, time to first COPD exacerbation event, post-BD FEV1, respiratory symptoms, disease impact, safety and tolerability	• FPCD: Q1 2023 • Data anticipated: 2025

As of 25 April 2024.
Appendix: Glossary.

AZD4041 (orexin 1 receptor antagonist)

Opioid use disorder

Trial	Population	Patients	Design	Endpoints	Status
Phase I NCT05587998 Partnered (National Institute on Drug Abuse)	Healthy recreational opioid users	36	• Randomised, double-blind, placebo-controlled, fixed sequence trial	• Primary endpoint: change in respiratory parameters	• FPCD: Q3 2022 • LPCD: Q2 2023 • Data readout: Q3 2023 • Primary endpoint met

As of 25 April 2024.
Appendix: Glossary.

MEDI0618 (PAR2 antagonist mAb)

Osteoarthritis pain, migraine prevention

Trial	Population	Patients	Design	Endpoints	Status
Phase I **NCT05714254**	Healthy volunteers	48	• Randomised, double-blind, placebo-controlled MAD trial • Arm 1: MEDI0618 i.v. or placebo • Arm 2: MEDI0618 s.c. or placebo	• Primary endpoints: safety, tolerability and PK parameters	• FPCD: Q4 2022 • LPCD: Q3 2023 • Data readout: Q1 2024

As of 25 April 2024.
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)	138	• Randomised, double-blind, placebo-controlled trial • Early PK cohort • Arm 1: TAK-341/MEDI1341 i.v. • Arm 2: placebo i.v. • Main cohort • Arm 3: TAK-341/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 • Data anticipated: 2025

As of 25 April 2024.
Appendix: Glossary.

MEDI1341 (alpha-synuclein mAb)

Parkinson’s disease

Trial	Population	Patients	Design	Endpoints	Status
Phase I **NCT04449484** **Partnered (Takeda)**	Parkinson's disease	25	• MAD trial • Arm 1: MEDI1341 i.v. • Arm 2: placebo i.v. • US only	• Primary endpoints: safety and tolerability • Secondary endpoints: PK and PD parameters	• FPCD: Q3 2020 • LPCD: Q3 2021 • Data readout: Q4 2022

As of 25 April 2024.
Appendix: Glossary.

MEDI7352 (NGF TNF bispecific mAb)

Osteoarthritis pain

Trial	Population	Patients	Design	Endpoints	Status
Phase IIb NCT04675034	Painful osteoarthritis of the knee	350	• MAD trial • Arm 1: MEDI7352 s.c. • Arm 2: placebo s.c. • Global – 7 countries	• Primary endpoint: dose response • Secondary endpoints: safety, tolerability, PK and PD parameters, ADA	• FPCD: Q1 2021 • LPCD: Q3 2022 • Data readout: Q4 2023
Phase IIa NCT03755934	Painful diabetic neuropathy	107	• MAD trial • Arm 1: MEDI7352 i.v. • Arm 2: placebo i.v. • Europe only	• Primary endpoint: dose response • Secondary endpoints: safety, tolerability, PK and PD parameters	• FPCD: Q4 2018 • LPCD: Q1 2023 • Data readout: Q4 2023

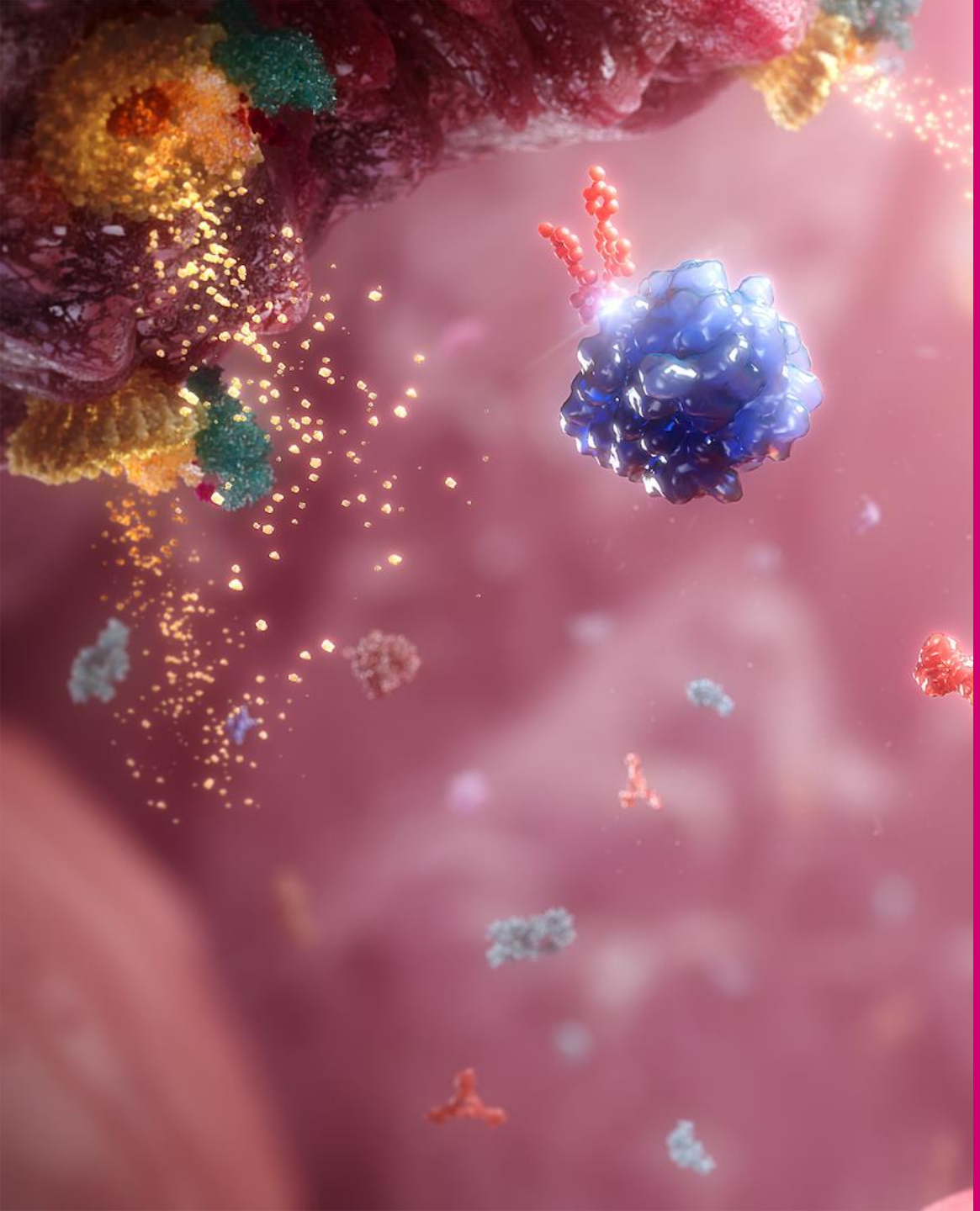
As of 25 April 2024.
Appendix: Glossary.

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 study 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 ose of licensed mRNA vaccinein 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 • Data anticipated: >2025

As of 25 April 2024.
Appendix: Glossary.



Rare Disease:
approved medicines and late-stage development

AstraZeneca

Koselugo (selumetinib, MEK inhibitor)

Neurofibromatosis type 1, solid tumours

Trial	Population	Patients	Design	Endpoints	Status
Phase III **KOMET** **NCT04924608**	Adult age ≥18 years with NF1 who have symptomatic, inoperable PN Available baseline chronic target PN pain score	146	• 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 • Secondary endpoint: change in baseline of chronic PN-pain intensity on Koselugo vs. placebo	• FPCD: Q4 2021 • Data anticipated: H2 2024
Phase I/II **SPRINKLE** **NCT05309668**	Paediatric (age 1 to 6 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 • Data anticipated: H2 2024
Phase I **China PK/Safety/Efficacy** **NCT04590235**	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
Phase I **Food Effect/GI Tolerability** **NCT05101148**	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 anticipated: >2025

As of 25 April 2024.
Appendix: Glossary.

Ultomiris (anti-C5 mAb)

Haematology, nephrology

Trial	Population	Patients	Design	Endpoints	Status
Phase III **ALXN1210-TM-313** **NCT04543591**	Thrombotic microangiopathy-associated haematopoietic stem cell transplant	106	• Arm 1: *Ultomiris* Q8W • Arm 2: placebo	• Primary endpoint: TMA response • Secondary endpoints: time to TMA response, TMA relapse	• FPCD: Q4 2020 • Data anticipated: 2025
Phase III **ALXN1210-TM-314** **NCT04557735**	Paedeatric thrombotic microangiopathy-associated haematopoietic stem cell transplant	40	• 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 • Data anticipated: 2025
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: >2025
Phase III **ICAN** **NCT06291376**	Immunoglobulin A nephropathy	450	• 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: change from baseline in proteinuria based on 24-hour UPCR at Weeks 10, 26, 34, 50, and 106 and change from baseline in eGFR at Weeks 34, 50, and 106	• Initiating • Data anticipated: >2025
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 • Data anticipated: >2025 • Primary endpoint met (IgAN cohort)

As of 25 April 2024.
Appendix: Glossary.

Ultomiris (anti-C5 mAb)

Neurology

Trial	Population	Patients	Design	Endpoints	Status
Phase III **ALXN1210-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 **ALXN1210-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 • Data anticipated: >2025

As of 25 April 2024.
Appendix: Glossary.

Voydeya (factor D inhibitor)

Haematology

Trial	Population	Patients	Design	Endpoints	Status
Phase III **ALPHA** **NCT04469465**	PNH with clinically significant EVH	86	• Arm 1: *Voydeya* + C5 Inhibitor • Arm 2: placebo + C5 Inhibitor	• Primary endpoint: change from baseline in haemoglobin at Week 12 • Secondary endpoint: percentage of participants with transfusion avoidance	• FPCD: Q1 2021 • Data readout: Q3 2022 • Primary endpoint met
Phase III **ALXN2040-PNH-303** **NCT05389449**	PNH	100	• Arm 1: *Voydeya* together with background C5 inhibitor therapy	• Primary endpoint: participants experiencing TEAEs and serious TEAEs	• FPCD: Q4 2022 • Data anticipated: >2025

As of 25 April 2024.
Appendix: Glossary.

acoramidis (ALXN2060)

ATTR-CM

Trial	Population	Patients	Design	Endpoints	Status
Phase III **ALXN2060-TAC-302** **NCT04622046**	ATTR-CM	22	• Arm 1: 800mg acoramidis 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 25 April 2024.
Appendix: Glossary.

ALXN2220 (NI006, TTR depleter)

Amyloidosis

Trial	Population	Patients	Design	Endpoints	Status
Phase III DepleTTR-CM NCT06183931	ATTR-CM	1000	• Arm 1: ALXN2220 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 endpoints: all-cause mortality and total CV events	• FPCD: Q1 2024 • Data anticipated: >2025

As of 25 April 2024.
Appendix: Glossary.

anselamimab (CAEL-101, fibril-reactive mAb)

AL amyloidosis

Trial	Population	Patients	Design	Endpoints	Status
Phase III CAEL101-302 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: time from first dose of trial drug until death or end of trial • Secondary endpoint: change in distance walked during a six-minute walk test and quality of life measures	• FPCD: Q4 2020 • Data anticipated: 2025
Phase III CAEL101-301 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: time from first dose of trial drug until death or end of trial • Secondary endpoint: change in distance walked during a six-minute walk test and quality of life measures	• FPCD: Q1 2021 • Data anticipated: 2025
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 anticipated: H1 2024

As of 25 April 2024.
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 of efzimfotase alfa ither 20mg, 35mg or 50mg Q2W via s.c. injection for 24 weeks	• Primary endpoint: change from baseline in 6MWT at Day 169	• FPCD: Q2 2024 • Data anticipated: >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: number of participants TEAEs	• Initiating • Data anticipated: >2025
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 • Data readout: Q4 2022 • Primary endpoint met

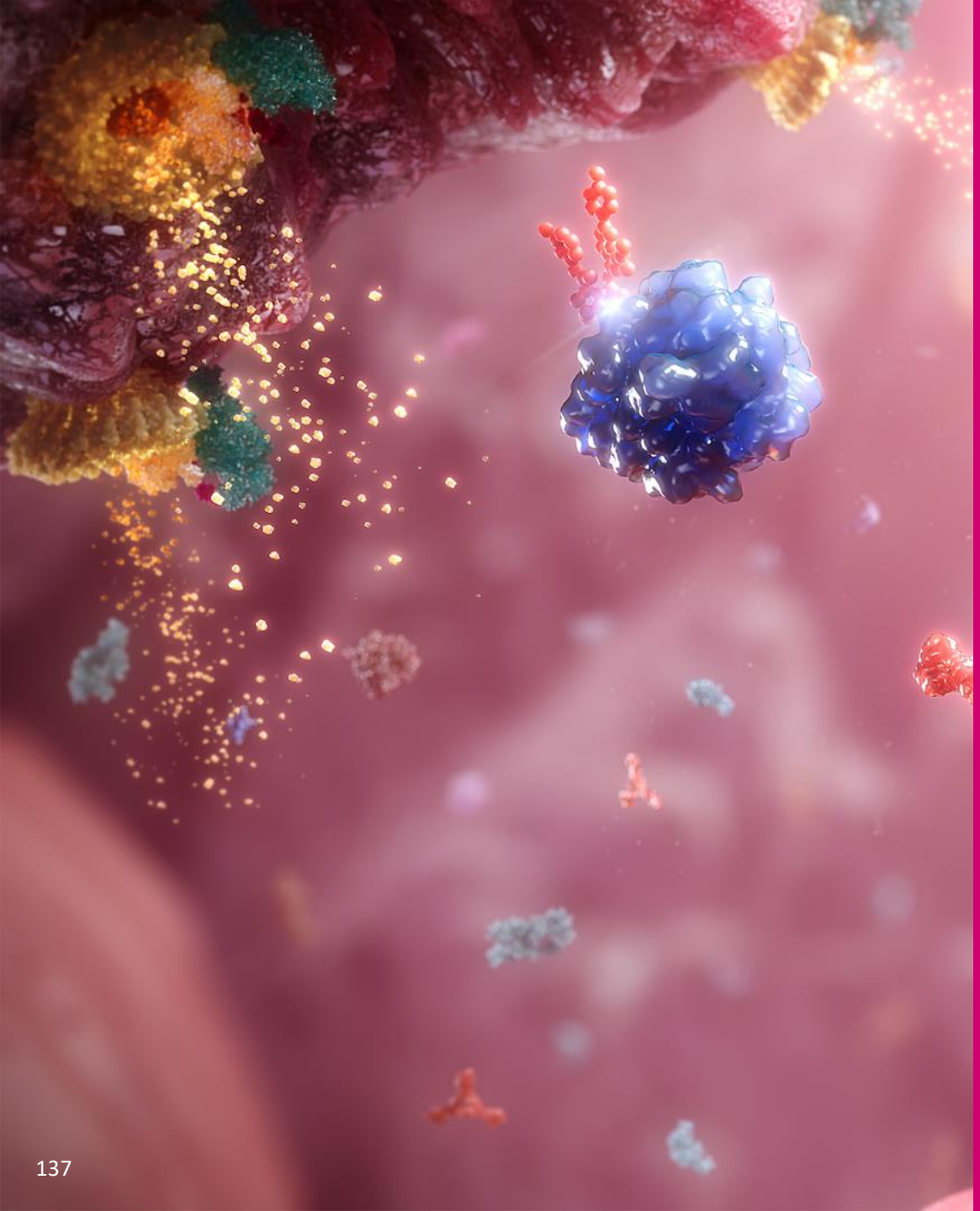
As of 25 April 2024.
Appendix: Glossary.

gefurulimab (ALXN1720, anti-C5 humanised bispecific heavy-chain antibody)

Neurology, nephrology

Trial	Population	Patients	Design	Endpoints	Status
Phase III **ALXN1720-MG-301** **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	• FPCD: Q4 2022 • Data anticipated: >2025
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 • Data readout: Q3 2023

As of 25 April 2024.
Appendix: Glossary.



Rare Disease:
early-stage development

AstraZeneca

danicopan (factor D inhibitor)

Ophthalmology

Trial	Population	Patients	Design	Endpoints	Status
Phase II **ALXN2040-GA-201** **NCT05019521**	Geographic atrophy	365	• Arms 1-3: danicopan dosed at 100mg-400mg QD • Arm 4: placebo	• Primary endpoint: mean rate of change from baseline at Week 52 in the square root of total GA lesion area in the trial eye as measured by FAF	• FPCD: Q3 2021 • Data anticipated: 2025

As of 25 April 2024.
Appendix: Glossary.

ALXN1910 (next-generation TNSALP ERT)

Bone metabolism

Trial	Population	Patients	Design	Endpoints	Status
Phase I **ALXN1910-HV-101** **NCT05307978**	Healthy adults	48	• Randomised, placebo-controlled SAD	• Primary endpoint: safety	• FPCD: Q2 2022 • Data readout: Q2 2023

As of 25 April 2024.
Appendix: Glossary.

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	• Primary endpoint: safety and tolerability • Secondary endpoints: PK/PD parameters	• FPCD: Q2 2023 • Data anticipated: H2 2024

As of 25 April 2024.
Appendix: Glossary.

ALXN2030 (siRNA targeting complement C3)

Nephrology

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

As of 25 April 2024.
Appendix: Glossary.

ALXN2080 (factor D inhibitor)

Complement-mediated disease

Trial	Population	Patients	Design	Endpoints	Status
Phase I **ALXN2080-HV-101** **NCT05428696**	Healthy volunteers	90	• SAD/MAD trial	• Primary endpoints: safety and tolerability, PK and PD parameters	• FPCD: Q3 2022 • Data readout: Q3 2023

As of 25 April 2024.
Appendix: Glossary.

vemircopan (ALXN2050, factor D inhibitor)

Haematology, nephrology, neurology

Trial	Population	Patients	Design	Endpoints	Status
Phase II **ALXN2050-gMG-201** **NCT05218096**	Generalised myasthenia gravis	70	• Arm 1: vemircopan 180mg • Arm 2: vemircopan 120mg • Arm 3: placebo followed by vemircopan	• Primary endpoint: MG-ADL total score reduction of ≥2 points in any 4 consecutive weeks during the first 8 weeks and who did not receive rescue therapy	• FPCD: Q2 2022 • Trial discontinued due to lack of efficacy
Phase II **ALXN2050-NEPH-201** **NCT05097989**	Lupus nephritis or immunoglobulin A nephropathy	126	• Arm 1 – LN cohort: vemircopan 180mg • Arm 2 – LN cohort: vemircopan 120mg • Arm 3 – LN cohort: placebo • Arm 4 – IgAN cohort: vemircopan 180mg • Arm 5 – IgAN cohort: vemircopan 120mg • Arm 6 – IgAN cohort: placebo	• Primary endpoint (both cohorts): percentage change in proteinuria from baseline to Week 26	• FPCD: Q3 2022 • Data anticipated: >2025
Phase I **ALXN2050-HV-109** **NCT05259085**	Impaired hepatic function	36	• Arm 1: mild IHF, 120mg vemircopan BID orally on Days 1 through 3, 120mg orally on the morning of Day 4 • Arm 2: moderate IHF, 120mg vemircopan BID orally on Days 1 through 3, 120mg orally on the morning of Day 4 • Arm 3: severe IHF, 120mg vemircopan BID orally on Days 1 through 3, 120mg orally on the morning of Day 4 • Arm 4: healthy control, 120mg vemircopan BID orally on Days 1 through 3, 120mg orally on the morning of Day 4	• Primary endpoint (Arm 1): AUC0-12 of plasma vemircopan after steady-state • Primary endpoint (Arm 2): AUCt of plasma vemircopan after steady-state • Primary endpoint (Arm 3): Cmax,ss of vemircopan • Primary endpoint (Arm 4): Tmax,ss following vemircopan	• FPCD: Q2 2022 • Data anticipated: H2 2024

As of 25 April 2024.
Appendix: Glossary.

NEXT

Glossary – 1 of 4

14C	Carbon 14
1L, 2L, 3L	1st-, 2nd- or 3rd-line
5-FU	5-fluorouracil
6MWT	6-minute walk test
A2AR	Adenosine A2A receptor
ACQ	Asthma Control Questionnaire
ACR	American College of Rheumatology Response Scoring System
ADA	Anti-drug antibody
ADC	Antibody-drug conjugate
ADP	Adenosine diphosphate
AE	Adverse event
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
anti-FRα	Anti-folate receptor alpha
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
AS	Albuterol sulfate
ASO	Antistreptolysin O
ATM	Ataxia telangiectasia mutated kinase
ATR	Ataxia telangiectasia and Rad3-related protein
ATTR	Transthyretin amyloidosis
ATTR-CM	Transthyretin amyloid cardiomyopathy
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
BTK	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
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
CE	Clinically evaluable
CHD	Coronary heart disease
Chemo	Chemotherapy
CHF	Chronic heart failure
cHL	Classic Hodgkin lymphoma
CKD	Chronic kidney disease
CLD	Chronic lung disease
CLDN18.2	Claudin 18.2
CLL	Chronic lymphocytic leukaemia
cm	Centimetre
CMAX	Maximum observed plasma concentration
cMET	Tyrosine-protein kinase mesenchymal epithelial transition factor
CMML	Chronic myelomonocytic leukaemia
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
CSA-AKI	Cardiac surgery-associated acute kidney injury
CTC	Circulating tumour cell
CTCAE	Common Terminology Criteria for Adverse Events
ctDNA	Circulating tumor DNA
CTLA-4	Cytotoxic T-lymphocyte-associated antigen-4

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Appendix: Glossary.

Glossary – 2 of 4

CTx	Chemotherapy
CV	Cardiovascular
CVOT	Cardiovascular outcomes trial
CXCR2	C-X-C Motif chemokine receptor 2
CyBorD	Cyclophosphamide, bortezomib and dexamethasone
Dato-DXd	Datopotamab deruxtecan
DCR	Disease control rate
DDFS	Distant disease-free survival
DDI	Drug-drug Interaction
dECG	Differentiated electrocardiogram
DFS	Disease-free survival
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
DoCR	Durability of complete response
DoR	Duration of response
DPB	Disease progression in bone
DPI	Dry powder inhaler
DRFI	Disease recurrence-free interval
DSQ	Dysphagia Symptom Questionnaire
DXA	Dual energy X-ray absorptiometry
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
EoE	Eosinophilic oesophagitis
ER	Estrogen receptor
ER+	Estrogen receptor-positive
ERK	Extracellular signal-regulated kinase
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
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-lipooxygenase 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
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
GMFR	Geometric mean fold rise
gMG	Generalised myasthenia gravis
GMT	Geometric mean titer
GPC3	Glypican 3
GPC3-positive	Glypican 3-positive
GPRC5D	G protein-coupled receptor, class C, group 5, member D
H1	H1-antihistamine
hADME	Human mass balance
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
HFO	Hydrofluoro-olefins
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
HLA-A*02:01	Human leukocyte antigen serotype within the HLA-A serotype group
HNSCC	Head and neck squamous-cell carcinoma
HPD	Hyperprogressive disease
HPDD	Highest protocol-defined dose
HPF	High-power field
HPP	Hypophosphatasia
HR+	Hormone receptor-positive
HRD	Homologous recombination deficiency
HRD+	Homologous recombination deficiency-positive
HR-low	Hormone receptor-low
HRRm	Homologous recombination repair-mutated
HSD17B13	Hydroxysteroid 17-beta dehydrogenase 13
HVPG	Hepatic venous pressure gradient
i	Inhibitor
i.m.	Intramuscular
i.v.	Intravenous
IA	Investigator-assessed
ICR	Independent central review
ICS	Inhaled corticosteroid
ICS-LABA	Inhaled corticosteroid long-acting beta-agonists
ICU	Intensive care unit

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Appendix: Glossary.

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IDFS	Invasive disease-free survival
IgAN	Immunoglobulin A nephropathy
IHF	Impaired hepatic function
IL	Interleukin
IL-12	Interleukin-12
IL-33	Interleukin-33
IL-5R	Interleukin-5 receptor
IMAC-TIS	International Myositis Assessment and Clinical Studies-Total Improvement Score
INV	Investigator review
IO	Immuno-oncology
IPFS	Invasive progression-free survival
IRAK4	Interleukin-1 receptor-associated kinase 4
IRC	Independent review committee
ISS	Investigator-sponsored studies
ISS7	Itch-severity score (weekly)
ITT	Intent-to-treat
JAK-1	Janus kinase 1
KCCQ	ansas City Cardiomyopathy Questionnaire
kg	Kilogram
Ki67	Antigen Kiel 67
LAAB	Long-acting antibody
LABA	Long-acting beta agonist
LAMA	Long-acting muscarinic agonist
LCAT	Lecithin-cholesterol acyltransferase
LDH	Lactate dehydrogenase
LDL-C	Low-density lipoprotein cholesterol
LICA	Ligand-conjugated ASO
LIF	Low-density lipoprotein cholesterol
LN	Lupus nephritis
LOS	Length of stay
LPCD	Last patient commenced dosing
LSD	Last subject dosed
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
MCC	Mucociliary clearance
MCL	Mantle cell lymphona
mCRPC	Metastatic castrate-resistant prostate cancer
MDI	Metered-dose inhaler
MDS	Myelodysplastic syndrome
MEK	Mitogen-activated protein kinase
MET	Mesenchymal epithelial transition factor
mFOLFOX	Modified folinic acid, fluorouracil and oxaliplatin
mg	Milligram
MG-ADL	Myasthenia Gravis-Activities of Daily Living
MI	Myocardial infarction
MMAE	Monomethyl auristatin E
MMT	Mixed meal test
mPFS	Median progression-free survival
MPO	Myeloperoxidase
mPR	Major pathological response
MR	Mineralocorticoid receptor
MRD-negative	Minimal residual disease-negative
MRI	Magnetic resonance imaging
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
nAb	Neutralising antibody
NaC	Sodium channel
NAFLD	Non-alcoholic fatty liver disease
NASH	Non-alcoholic fatty liver disease
NCFB	Non-cystic fibrosis bronchiectasis
NCI	National Cancer Institute
NCPV	Noncalcified plaque volume
NF1	Neurofibromatosis type 1
NF1-PN	Neurofibromatosis type 1 with plexiform neurofibromas
ng	Next-generation
NGF	Nerve growth factor
NHA	New hormonal agents
NHL	Non-Hodgkin's lymphoma
NIH	National Institute of Health
NKTCL	Extranodal natural killer T-cell lymphoma
NMOSD	Neuromyelitis optica spectrum disorder
NRG	National Clinical Trials Network in Oncology
NSCLC	Non-small cell lung cancer
OBD	Optimal biological dose
OCS	Oral corticosteroid
OD	Once daily
OGTT	Oral glucose tolerance test
OR	Objective response
ORR	Overall response rate
OS	Overall surival
PA	Primary aldosteronism
PALB2m	Partner and localizer of BRCA2-mutated
PAR2	Protease-activated receptor 2
PARP	Poly ADP ribose polymerase
PARP-1sel	Poly ADP ribose polymerase-1 selective
PASI	Psoriasis area severity index
PBD	Pyrrolobenzodiazepine
PCD	Plasma cell dyscrasia
pCR	Pathological complete response
PCSK9	Proprotein convertase subtilisin/kexin type 9
PD	Pharmacodynamics
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 3 kinase catalytic alpha gene
PK	Pharmacokinetic
PLL	Prolymphocytic leukaemia
pMDI	Pressurised metered-dose inhaler
PN	Plexiform neurofibroma
PNH	Paroxysmal nocturnal haemoglobinuria
POC	Proof of concept
POM	Proof of mechanism

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Appendix: Glossary.

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post-BD	Post-bronchodilator
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
PSC	Pulmonary sarcomatoid carcinoma
PSMA	Prostate-specific membrane antigen
PSR	Platinum-sensitive relapsed
PTCL	Peripheral T-cell lymphoma
PTEN	Phosphatase and tensin homolog gene
PVR	Pulmonary vascular resistance
Q1W	Every week
Q4W	Every four weeks
Q8W	Every eight weeks
QD	Once daily
QID	Four times per day
QOD	Evey 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/R	Relapsed/refractory
RA	Rheumatoid arthritis
RAAS	Renin-angiotensin-aldosterone system
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.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
SGLT2	Sodium-glucose transport protein 2
SGRM	Selective glucocorticoid receptor modulator
SGRQ	Saint George Respiratory Questionnaire
siRNA	Small interfering ribonucleic acid
SJC	Swollen joint count
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
STAT3	Signal transducer and activator of transcription 3
sUA	Serum uric acid
T2DM	Type 2 diabetes mellitus
T300	*Imfinzi* plus *Imjudo*
T790M	Threonine 790 substitution with methionine
TACE	Transarterial chemoembolization
tBRCAm	Tumour (somatic) BRCA-mutated
TCR-T	T-cell receptor therapy
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
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 p53
TP53 R175H	Tumour protein p53 with arginine at position 175 replaced with histidine
TPS	Tumour proportion score
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
UACR	Urine albumin creatinine ratio
UK	United Kingdom
u-LTE4	Urinary leukotriene E4
UMEC	Umeclidinium
UPCR	Urine protein creatinine ratio
URAT1	Uric acid transporter 1
US	United States
VEGF	Vascular endothelial growth factor
VHH	Single domain antibody
XELOX	Oxaliplatin and capecitabine
γH2AX	H2A histone family member X phosphorylated on serine 139

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Appendix: Glossary.