Clinical trials appendix

Q3 2021 results update

Movement since Q2 2021 update

New to Phase I	New to Phase II	New to Pivotal trial	New to registration
NME **AZD2936** PD1/TIGIT bispecific mAb solid tumours **AZD7789** PD1/TIM3 bispecific mAb solid tumours **AZD5462** Relaxin mimetic cardiovascular disease **AZD4604** Inhaled JAK1 asthma **ALXN1850** subcutaneous next generation asfotase alfa for Hypophosphatasia	**NME** **AZD5305** PARP1 solid tumours **Additional indication** **danicopan (ALXN2040)** oral factor D inhibitor for geographic atrophy	**NME** ***Orpathys*[#] + *Imfinzi*[#] SAMETA** MET inhibitor + PD-L1 mAb 1st-line papillary rencell carcinoma **Additional indication** **camizestrant (AZD9833) + CDK4/6i SERENA-6** selective oestrogen receptor degrader + CDK4/6 inhibitors 1st-line HR+ HER2- ESR1m breast cancer ***Saphnelo*[#] (anifrolumab)** Type I IFN receptor mAb systemic lupus erythematosus (subcutaneous) **Lifecycle management** ***Imfinzi* + EV + tremelimumab VOLGA** PD-L1 + nectin-4 targeting ADC +/- CTLA4 muscle invasive bladder cancer ***Imfinzi* MERMAID-2** PD-L1 mAb stage II-III premetastatic NSCLC ***Lynparza*[#] MONO-OLA1** PARP inhibitor 1st-line BRCAwt ovarian cancer ***Fasenra* MAHALE** IL5R mAb non-cystic fibrosis bronchiectasis ***Lokelma* STABILIZE-CKD** potassium binder hyperkalaemia in CKD ***Ultomiris* (ALXN1210)** Complement-Mediated Thrombotic Microangiopathy	**NME** **AZD7442 [US][1]** COVID-19 LAAB combination prevention and treatment of COVID-19

Phase progressions based on first patient dose achievement.

[1] Prophylaxis FDA EUA submission [#] Partnered and/or in collaboration

Movement since Q2 2021 update

Removed from Phase I	Removed from Phase II	Removed from Phase III	Removed from registration
NME **MEDI5395** rNDV GMCSF solid tumours	**NME** **AZD9567** oral SGRM chronic inflammatory diseases ***Imfinzi*[#] + imaradenant + cabazitaxel** PD-L1 mAb + A2aR inhibitor + chemotherapy prostate cancer ***Imfinzi*[#] + MEDI0457[#]** PD-L1 mAb + DNA HPV vaccine HNSCC **verinurad** URAT1 inhibitor CKD / heart failure with a preserved ejection fraction	**Lifecycle Management** ***Calquence* ELEVATE-RR[2]** BTK inhibitor relapsed/refractory chronic lymphocytic leukaemia, high risk ***Ultomiris* (ravulizumab) CHAMPION-ALS** amyotrophic lateral sclerosis	**NME** ***Saphnelo*[#] (anifrolumab) TULIP [US & JP][1]** Type I IFN receptor mAb systemic lupus erythematosus

Phase progressions based on first patient dose achievement.
[1] Approved [2] Not registrational [#] Partnered and/or in collaboration

Q3 2021 Oncology new molecular entity[1] pipeline

Phase I
15 Projects

- AZD0466# BCL2/xL haematological tumours
- AZD1390 ATM glioblastoma
- AZD2936 PD1/TIGIT bispecific mAb solid tumours
- AZD5991 MCL1 haematological malignancies
- AZD4573 CDK9 haematological malignancies
- AZD7648# DNAPK solid and haematological tumours
- AZD7789 PD1/TIM3 bispecific mAb solid tumours
- AZD8701# FOXP3 solid tumours
- *Imfinzi*#+adavosertib# PD-L1+Wee1 solid tumours
- IPH5201# CD39 solid tumours
- MEDI1191 IL12 mRNA solid tumours
- *Imfinzi*+selumetinib# PD-L1+MEK solid tumours
- MEDI9253 rNDV IL12 solid tumours
- MEDI5752+lenvatinib PD-1/CTLA-4+VEGF advanced renal cell carcinoma
- *Tagrisso* combo# TATTON EGFR+MEK/MET advanced EGFRm NSCLC

Phase II
16 Projects

- adavosertib# Wee1 ovarian / uterine serous / solid tumours
- AZD2811 nanoparticle Aurora B solid tumours
- AZD5305 PARP1 solid tumours
- camizestrant (AZD9833) SERD ER+ breast
- capivasertib# AKT prostate
- ceralasertib ATR solid tumours
- *Imfinzi* (platform) HUDSON PD-L1+multiple novel ONC therapies post IO NSCLC
- *Imfinzi*# (platform) COAST PD-L1+multiple novel ONC therapies NSCLC
- *Imfinzi*# (platform) NeoCOAST PD-L1+multiple novel ONC therapies NSCLC
- Imfinzi#+Lynparza# ORION PD-L1+PARP 1L mNSCLC
- *Imfinzi*#+monalizumab# PD-L1+NKG2a solid tumours
- *Imfinzi*+FOLFOX+bevacizumab (platform) COLUMBIA1 PD-L1+chemo + VEGF+multiple novel ONC therapies 1L MSS-CRC
- MEDI5752 PD-1/CTLA-4 solid tumours
- oleclumab+chemo or *Imfinzi*#+ oleclumab+chemo CD73+chemo or PD-L1+CD73+chemo pancreatic
- Post-1L *Tagrisso* (platform) ORCHARD EGFR+multiple novel ONC therapies EGFRm NSCLC
- *Tagrisso*+savolitinib# SAVANNAH EGFR+MET advanced EGFRm NSCLC

Phase III
15 Projects

- camizestrant+CDK4/6i SERENA-6 SERD+CDK4/6 1L HR+ HER2- ESR1m breast cancer
- camizestrant+palbociclib SERENA-4 SERD+CDK4/6 1L HR+ HER2- breast cancer
- capivasertib#+abiraterone CAPItello-281 AKT+abiraterone PTEN deficient metastatic hormone sensitive prostate cancer
- capivasertib#+chemo CAPItello-290 AKT+chemotherapy mTNBC 1L
- capivasertib#+fulvestrant+palbociclib CAPItello-292 AKT + fulvestrant + CDK4/6 HR+ breast 1L
- capivasertib#+fulvestrant CAPItello-291 AKT+fulvestrant locally-advanced (inoperable) or metastatic breast
- datopotamab deruxtecan# TROPION-Lung01 TROP2 2L+ NSCLC without actionable mutation
- *Imfinzi*#+/-tremelimumab+chemo POSEIDON PD-L1+/-CTLA-4+SoC 1L NSCLC
- *Imfinzi*#+/-tremelimumab+CRT ADRIATIC PD-L1+/-CTLA-4+CRT LS-SCLC
- *Imfinzi*#+tremelimumab HIMALAYA PD-L1+CTLA-4 1L HCC
- *Imfinzi*#+tremelimumab+SoC NILE PD-L1+CTLA-4+SoC 1L urothelial cancer
- *Lynparza*#+*Imfinzi*# DuO-E PARP+PD-L1 1L endometrial cancer
- *Lynparza*#+*Imfinzi*#+bevacizumab DuO-O PARP+PD-L1+VEGF 1L ovarian
- monalizumab#+cetuximab (INTERLINK-1) NKG2a+EGFR 2L+ relapsed metastatic HNSCC
- *Orpathys*#+*Imfinzi*# SAMETA MET+PD-L1 1L papillary renal cell carcinoma

Under review
0 Projects

Phase progressions based on first patient dose achievement.
[1] Includes novel combinations and additional indications for assets where the lead is not yet launched.
Partnered and/or in collaboration; ¶ Registrational Phase II/III trial

Precision medicine approach being explored

Q3 2021 Oncology life-cycle management[1] pipeline

Phase I
1 Project

- *Enhertu*# (platform) DESTINY-Breast08 ADC breast

Phase II
7 Projects

- *Enhertu*# (platform) DESTINY-Breast07 ADC breast
- *Enhertu*# DESTINY-Lung01 ADC NSCLC
- *Enhertu*# DESTINY-PanTumor01 HER2 targeting ADC HER2-expressing solid tumours
- *Enhertu*# DESTINY-PanTumor02 HER2 targeting ADC HER2-expressing solid tumours
- *Imfinzi*# (platform) BEGONIA PD-L1 1L mTNBC
- *Imfinzi*# (platform) MAGELLAN PD-L1 1L mNSCLC
- *Lynparza*# (basket) MK-7339-002 / LYNK002 PARP HRRm cancer

Phase III
33 Projects

- *Calquence*# ECHO BTK inhibitor 1st line MCL
- *Calquence*+R-CHOP ESCALADE BTK+R-CHOP 1L DLBCL
- *Calquence*#+venetoclax+obinutuzumab BTK+BCL-2+anti-CD20 1st line CLL
- *Enhertu*# DESTINY-Breast02 ADC breast
- *Enhertu*# DESTINY-Breast05 ADC breast
- *Enhertu*# DESTINY-Breast03 ADC breast
- *Enhertu*# DESTINY-Breast04 ADC breast
- *Enhertu*# DESTINY-Breast09 HER2 targeting ADC HER2+ breast cancer 1L
- *Enhertu*# DESTINY-Breast06 ADC breast
- *Enhertu*# DESTINY-Gastric04 HER2 targeting ADC HER2+ gastric cancer 2L
- *Imfinzi*# PEARL PD-L1 1L metastatic NSCLC
- *Imfinzi* MERMAID-2 PD-L1 stage II-III premetastatic NSCLC
- *Imfinzi*# CALLA PD-L1 adj. locally-advanced cervical cancer
- *Imfinzi*# POTOMAC PD-L1 non muscle invasive bladder cancer
- *Imfinzi*# post-SBRT PACIFIC-4 PD-L1 post-SBRT stage I/II NSCLC
- *Imfinzi*#+CRT KUNLUN PD-L1+CRT locally-advanced esophageal squamous cell carcinoma
- *Imfinzi*#+Ctx MERMAID-1 PD-L1 stage II-III adjuvant NSCLC
- *Imfinzi*#+CRT PACIFIC-2 PD-L1+CRT NSCLC
- *Imfinzi*#+CRT PACIFIC-5 (China) PD-L1+CRT locally-advanced stage III NSCLC
- *Imfinzi*#+CTx NIAGARA PD-L1+CTx muscle invasive bladder cancer
- *Imfinzi*#+CTx neoadjuvant AEGEAN PD-L1+CTx locally-advanced stage II-III NSCLC
- *Imfinzi*#+CTx TOPAZ-1 PD-L1+CTx 1L biliary tract cancer
- *Imfinzi*#+VEGF+TACE EMERALD-1 PD-L1+VEGF+TACE locoregional HCC
- *Imfinzi*#+FLOT MATTERHORN PD-L1+CTx neo-adjuvant/adjuvant gastic cancer
- *Imfinzi*#+VEGF EMERALD-2 PD-L1+VEGF adjuvant HCC
- *Imfinzi* + EV +- treme VOLGA PD-L1 + nectin-4 targeting ADC +/- CTLA4 muscle invasive bladder cancer
- *Lynparza*# MONO-OLA1 PARP 1L BRCAwt ovarian cancer
- *Lynparza*# OlympiA PARP gBRCA adjuvant breast
- *Lynparza*+abiraterone# PROpel PARP+NHA prostate cancer
- *Lynparza*# LYNK-003 PARP platinum sensitive 1L colorectal cancer
- *Tagrisso* LAURA EGFRm locally-advanced unresectable NSCLC
- *Tagrisso*+chemo FLAURA2 EGFR+chemo 1L adv EGFRm NSCLC
- *Tagrisso*+/-CTx neoadjuvant NeoADAURA EGFR+/-CTx stage II/III resectable EGFRm NSCLC

Under review
0 Projects

Phase progressions based on first patient dose achievement.

[1] Includes significant life-cycle management projects and parallel indications for assets beyond Phase III

Partnered and/or in collaboration; ¶ Registrational Phase II/III trial

● Precision medicine approach being explored

Q3 2021 BioPharmaceuticals new molecular entity[1] pipeline

Phase I

13 Projects

- AZD0284 RORg psoriasis / respiratory
- AZD2373 Podocyte health nephropathy
- AZD3366 CD39L3 CV disease
- AZD4041# orexin 1 receptor antagonist opioid use disorder
- AZD4604 inhaled JAK1 asthma
- MEDI0618# PAR2 antagonist mAb osteoarthritis pain
- MEDI1814# amyloidβ alzheimer's disease
- AZD0449 Inhaled JAK inhibitor asthma
- AZD2693 nonalcoholic steatohepatitis
- AZD3427 Relaxin ThP CV disease
- AZD5462 Relaxin mimetic CV disease
- MEDI1341# alpha synuclein parkinson's disease
- MEDI8367 avb8 chronic kidney disease

Phase II

19 Projects

- AZD1402# inhaled IL-4Ra asthma
- AZD5718 FLAP coronary artery disease / CKD
- AZD8233 hypercholesterolemia cardiovascular
- brazikumab IL23 ulcerative colitis
- MEDI5884# cholesterol modulation cardiovascular
- MEDI7352 NGF/TNF OA pain / painful diabetic neuropathy
- *Saphnelo*# (anifrolumab) Type I IFN receptor lupus nephritis
- tezepelumab# COURSE TSLP COPD
- tozorakimab (MEDI3506) IL-33 AD / COPD / asthma / COVID-19
- *Zibotentan+Farxiga* ZENITH-CKD ETA antagonist+SGLT2 CKD
- AZD4831 MPO HFpEF
- AZD7986# DPP1 COPD
- AZD8601# VEGF-A cardiovascular
- AZD9977+*Farxiga* MR+SGLT2 HF with CKD
- cotadutide GLP-1/glucagon T2D / obesity / NASH / DKD
- MEDI6570 LOX-1 CV disease
- navafenterol# MABA COPD
- suvratoxumab α-Toxin Staphylococcus pneumonia
- tozorakimab (MEDI3506) IL-33 diabetic kidney disease

Phase III

6 Projects

- AZD2816¶# (next generation COVID-19 vaccine) SARS-CoV-2 prevention of COVID-19
- brazikumab¶ IL23 crohns disease
- nirsevimab# RSV mAb-YTE passive RSV immunisation
- PT027# ICS/SABA asthma
- *Saphnelo*# (anifrolumab) Type I IFN receptor SLE SC
- tezepelumab# WAYPOINT TSLP nasal polyps

Under review

2 Projects

- AZD7442 long-acting antibody combination COVID-19
- tezepelumab# NAVIGATOR TSLP severe uncontrolled asthma

Phase progressions based on first patient dose achievement.

[1] Includes novel combinations and additional indications for assets where the lead is not yet launched

Partnered and/or in collaboration; ¶ Registrational Phase II/III trial

● Precision medicine approach being explored

Q3 2021 BioPharmaceuticals life-cycle management[1] pipeline

Phase I
0 Projects

Phase II
3 Projects

- *Fasenra* ARROYO
  IL-5R chronic spontaneous urticaria
- *Fasenra* HILLIER
  IL-5R atopic dermatitis
- roxadustat#
  HIF-PH inhibitor chemo induced anaemia

Phase III
12 Projects

- *Breztri*
  LABA/LAMA/ICS asthma
- *Farxiga/Forxiga* DELIVER
  SGLT2 HFpEF
- *Fasenra* MAHALE
  IL-5R non-cystic fibrosis bronchiectasis
- *Fasenra* MESSINA
  IL-5R eosinophilic oesophagitis
- *Fasenra*# RESOLUTE
  IL-5R COPD
- *Lokelma* STABILIZE-CKD
  potassium binder hyperkalaemia in CKD
- *Farxiga/Forxiga* DAPA-MI
  SGLT2 prevention of HF and CV death following a myocardial infarction
- *Fasenra* FJORD
  IL-5R bullous pemphigoid
- *Fasenra* MANDARA
  IL-5R EGPA
- *Fasenra* NATRON
  IL-5R hypereosinophilic syndrome
- *Lokelma* DIALIZE-Outcomes
  potassium binder CV outcomes in patients on chronic haemodialysis with hyperkalaemia
- roxadustat#
  HIFPH anaemia MDS

Under review
1 Project

- *Fasenra*# OSTRO
  IL-5R nasal polyps

Phase progressions based on first patient dose achievement.
[1] Includes significant life-cycle management projects and parallel indications for assets beyond Phase III
Partnered and/or in collaboration; ¶ Registrational Phase II/III trial

● Precision medicine approach being explored

Q3 2021 Rare Disease pipeline[1]

Phase I

4 Projects

- ALXN1720
  subcutaneous anti-C5 bi-specific for generalised Myasthenia Gravis
- ALXN1820
  anti-properdin bi-specific for Haematology
- ALXN1830
  FcRn for Warm Autoimmune Haemolytic anaemia
- ALXN1850
  subcutaneous next generation asfotase alfa for Hypophosphatasia

Phase II

3 Projects

- ALXN2050
  oral factor D inhibitor for Paroxysmal Nocturnal Haemoglobinuria
- *Andexxa*
  Urgent Surgery
- danicopan (ALXN2040)
  oral factor D inhibitor for geographic atrophy

Phase III

9 Projects

- ALXN1840
  oral copper chelator for Wilson's disease (oral WD)
- acoramidis (ALXN2060)
  oral TTR stabilizer for Transthyretin Amyloid Cardiomyopathy (oral ATTR CM)
- CAEL-101
  AL amyloidosis
- danicopan (ALXN2040)
  Paroxysmal Nocturnal Haemoglobinuria-Extravascular Haemolysis (PNH-EVH)
- *Ultomiris*
  Complement-Mediated Thrombotic Microangiopathy (CMA-TMA)
- *Ultomiris*
  Generalised Myasthenia Gravis (gMG)
- *Ultomiris*
  Haemtopoietic Stem Cell Transplant–associated Thrombotic Microangiopathy (HSCT-TMA)
- *Ultomiris*
  Neuromyelitis Optica Spectrum Disorder (NMOSD)
- *Ultomiris*
  Subcutaneous administration Paroxysmal Nocturnal Haemoglobinuria (PNH) and atypical Haemolytic Uraemic Syndrome (aHUS)

Under review

0 Projects

Note. Projects with a precision medicine approach to be confirmed

Phase progressions based on first patient dose achievement.
[1] Includes new molecular entities and significant life-cycle management projects
Partnered and/or in collaboration; ¶ Registrational Phase II/III trial

Estimated key regulatory submission acceptances

	H2 2021	H1 2022	H2 2022	2022+		
NME	*Imfinzi* +/- tremelimumab NSCLC POSEIDON	*Koselugo* NF1 (China / Japan) SPRINT	acoramidis (ALXN2060) oral ATTR CM	datopotamab deruxtecan NSCLC TROPION-Lung01	monalizumab + cetuximab 2L+ relapsed mHNSCC INTERLINK-1	tezepelumab nasal polyposis WAYPOINT
		Imfinzi + tremelimumab HCC HIMALAYA	ALXN1840 oral WD	capivasertib + fulvestrant + palbociclib 1L locally advanced or mBC CAPItello-292	*Lynparza+Imfinzi*+ bevacizumab ovarian DUO-O	*Saphnelo* (anifrolumab) SLE subcutaneous
		Vaxzevria / COVID-19 Vaccine AstraZeneca (US)		capivasertib + fulvestrant 2L locally advanced or mBC CAPItello-291	*Lynparza + Imfinzi* endometrial cancer DUO-E	*Fasenra* severe asthma (China)
		PT027 asthma		capivasertb + CTx 1L mTNBC CAPItello-290	*Imfinzi* + tremelimumab + SoC urothelial NILE	brazikumab crohns disease INTREPID
		nirsevimab passive RSV immunisation		capivasertb + abiraterone PTEN deficient mHSPC CAPItello-281	*Imfinzi*+ tremelimumab + CRT LDS-SCLC ADRIATIC	danicopan (ALXN2040) oral factor D PNH-EVH
				camizestrant + palbociclib breast cancer SERENA-4	*Orpathys + Imfinzi* 1L PRCC SAMETA	CAEL-101 AL amyloidosis
				camizestrant + CDK4/6i 1L HR+ HER2- ESR1m breast cancer SERENA-6		
LCM	*Enhertu* DESTINY-Breast03	*Calquence* 1L CLL ELEVATE-TN (Japan)	*Enhertu* DESTINY-Breast02	*Calquence* + R-CHOP 1L DLBCL ESCALADE	*Imfinzi* + CRT neo-adjuvant/adjuvant gastric MATTERHORN	*Duaklir* Genuair COPD (China)
	Lynparza breast OLYMPIA	*Enhertu* DESTINY-Breast04	*Imfinzi* cervical CALLA	*Calquence* + venetoclax + obinutuzumab 1L CLL AMPLIFY	*Imfinzi* +CTx stage II-III adjuvant NSCLC MERMAID-1	*Farxiga* prevention of HF and CV death following a myocardial infarction DAPA-MI
	Lynparza + abiraterone prostate PROPEL	*Imfinzi* +CTx biliary tract TOPAZ-1	*Imfinzi* NSCLC PEARL	*Calquence* 1L MCL ECHO	*Imfinzi* stage II-III premetastatic NSCLC MERMAID-2	*Fasenra* bullous pemphigoid FJORD
	Ultomiris gMG	*Farxiga* HF (HFpEF) DELIVER	*Imfinzi* + CRT NSCLC PACIFIC-2	*Enhertu* DESTINY-Breast05	*Imfinzi* + chemo muscle invasive bladder NIAGARA	*Breztri/Trixeo* asthma KALOS, LOGOS
	Ultomiris s.c. PNH and aHUS	*Xigduo XR/Xigduo* type-2 diabetes (China)	*Imfinzi* + VEGF + TACE locoregional HCC EMERALD-1	*Enhertu* DESTINY-Breast06	*Imfinzi* post-SBRT NSCLC PACIFIC-4	*Fasenra* non-cystic fibrosis bronchiectasis MAHALE
			Fasenra eosinophilic esophagitis MESSINA	*Enhertu* DESTINY-Breast09	*Imfinzi* + CRT NSCLC PACIFIC-5 (China)	*Fasenra* EGPA MANDARA
			Ultomiris NMOSD	*Enhertu* DESTINY-Gastric04	*Imfinzi* non muscle invasive bladder POTOMAC	*Fasenra* HES NATRON
				Imfinzi neoadjuvant NSCLC AEGEAN	*Lynparza* 1L BRCAwt ovarian MONO-OLA1	*Fasenra* nasal polyps ORCHID (China / Japan)
				Imfinzi adjuvant NSCLC BR.31	*Tagrisso* + CTx EGFRm NSCLC FLAURA2	*Fasenra* COPD RESOLUTE
				Imfinzi + VEGF adjuvant HCC EMERALD-2	*Tagrisso* locally adv. unresectable NSCLC LAURA	*Lokelma* DIALIZE-Outcomes
				Imfinzi + CRT LA ESCC KUNLUN	*Lynparza* platinum sensitive 1L colorectal LYNK-003	*Lokelma* STABILIZE-CKD
				Imfinzi + EV +- treme VOLGA muscle invasive bladder cancer	*Tagrisso* stage II/III resectable EGFRm NSCLC NeoADAURA	roxadustat anemia in MDS
					Ultomiris CM-TMA	*Ultomiris* HSCT-TMA

Note. NME section includes novel combinations and additional indications for assets where the lead is not yet launched

Oncology BioPharmaceuticals Rare Disease

Designations in our pipeline

2 Accelerated approvals

- *Andexxa* Acute Major Bleed (US)
- *Calquence* MCL 1L (US)

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

- danicopan (ALXN2040) PNH-EVH (US)
- danicopan (ALXN2040) PNH-EVH (EU)
- *Calquence* CLL ELEVATE-TN, ASCEND (US)
- *Calquence* MCL 1L (US)
- *Enhertu* HER2+ breast 2L DESTINY-Breast03 (US)
- *Enhertu* HER2+/HER2low gastric 3L DESTINY-Gastric01 (US)
- *Enhertu* HER2+/HER2low gastric 3L DESTINY-Gastric01 (JP)[2]
- *Enhertu* HER2mut NSCLC 2L+ DESTINY-Lung01 (US)
- *Forxiga* CKD DAPA-CKD (US)
- *Koselugo* NFI type 1 SPRINT (US)
- nirsevimab RSV mAb-YTE MELODY-MEDLEY (US)
- nirsevimab RSV mAb-YTE MELODY-MEDLEY (CN)
- nirsevimab RSV mAb-YTE MELODY-MEDLEY (EU)[1]
- *Soliris* GBS (JP)[2]
- *Tagrisso* adjuvant NSCLC ADAURA (US)
- tezepelumab asthma NAVIGATOR (US)

8 Fast Track

- anifrolumab SLE (US)
- CAEL-101 AL amyloidosis (US)
- cotadutide NASH (US)
- *Fasenra* EG/EGE HUDSON (US)
- *Forxiga* CKD DAPA-CKD (US)
- *Forxiga* MI RRCT DAPA-MI (US)
- nirsevimab RSV mAb-YTE MELODY-MEDLEY (US)
- savratoxumab Staph HAP (US)

9 Priority Review

- *Brilinta* stroke THALES (US)
- *Calquence* MCL 1L (US)
- *Enhertu* HER2+/HER2low gastric 3L DESTINY-Gastric01 (US)
- *Forxiga* CKD DAPA-CKD (US)
- *Forxiga* CKD DAPA-CKD (JP)
- *Koselugo* NFI type 1 SPRINT (US)
- roxadustat chronic kidney disease (CN)
- *Tagrisso* adjuvant NSCLC (ADAURA) (US)
- tezepelumab Asthma NAVIGATOR (US)

28 Orphan

- ALXN1830 WAIHA (EU)
- danicopan (ALXN2040) PNH (US)
- danicopan (ALXN2040) PNH (EU)
- ALXN2050 PNH (US)
- ALXN2050 PNH (EU)
- ALXN1840 WD (US)
- ALXN1840 WD (EU)
- *Andexxa* Acute Major Bleed (JP)
- CAEL-101 AL amyloidosis (US)
- CAEL-101 AL amyloidosis (EU)
- *Calquence* CLL 1L (US)
- *Calquence* CLL 1L (EU)
- *Calquence* MCL 1L (US)
- *Enhertu* HER2+/HER2low gastric 3L DESTINY-Gastric01 (US)
- *Fasenra* EG/EGE HUDSON (US)
- *Fasenra* EGPA MANDARA (US)
- *Fasenra* EOE MESSINA (US)
- *Fasenra* HES NATRON (US)
- *Imfinzi* + CTx Biliary Tract 1L TOPAZ-1 (US)
- *Imfinzi* ± treme HCC 1L HIMALAYA (EU)
- *Imfinzi* + treme HCC 1L HIMALAYA (US)
- *Koselugo* NFI type 1 SPRINT (US)
- *Koselugo* NFI type 1 SPRINT (EU)
- *Koselugo* NFI type 1 SPRINT (JP)
- tezepelumab Eosinophilic Esophagitis (US)
- *Ultomiris* DM (US)
- *Ultomiris* HSCT-TMA (US)
- *Ultomiris* SC PNH (US)

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.

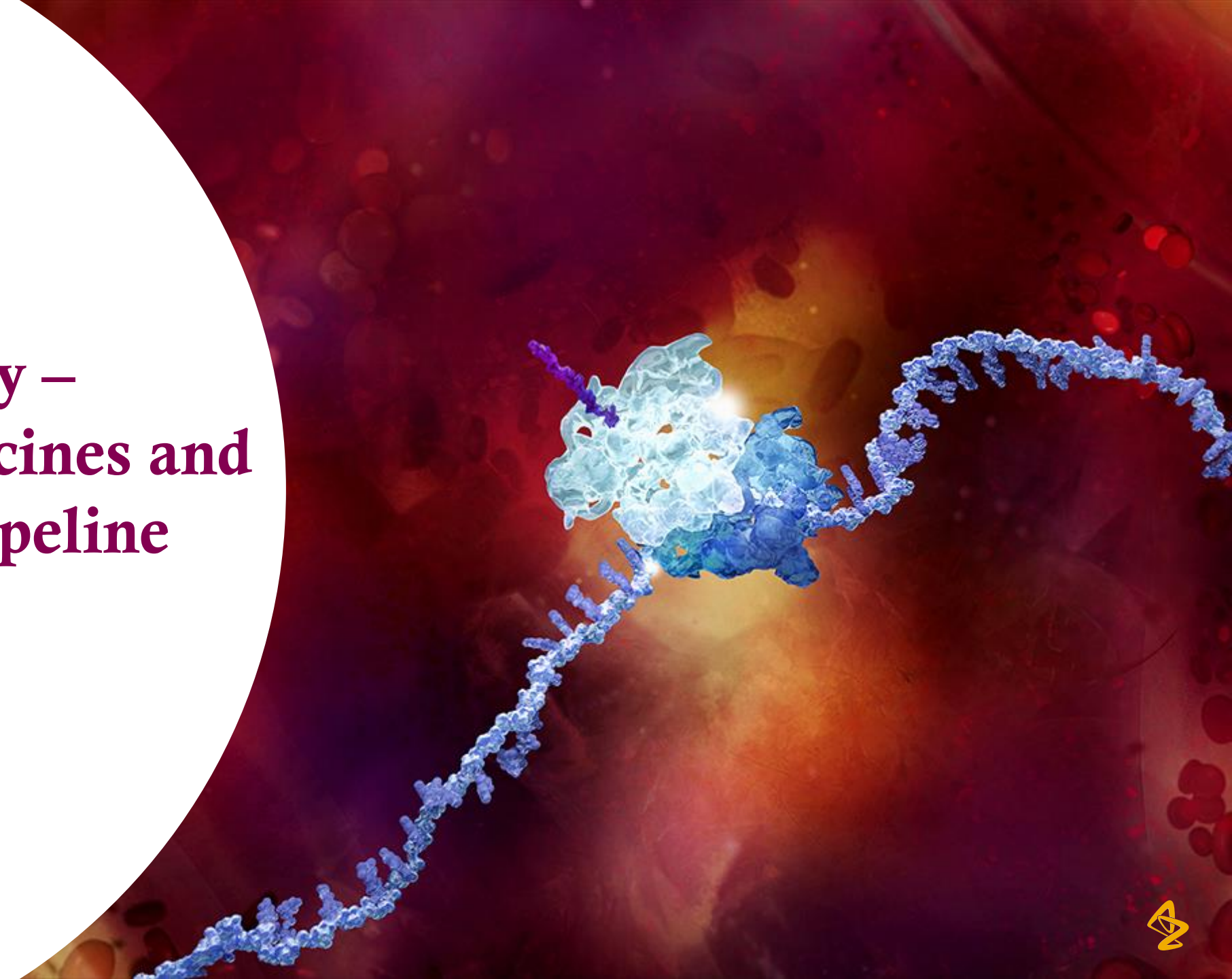
PRIORITY REVIEW DESIGNATION is the US FDA's goal to take action on an application within 6 months.

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. list excludes designations for projects which have launched in all applicable major markets

Oncology BioPharmaceuticals Rare Disease

Oncology – approved medicines and late-stage pipeline



Tagrisso (highly-selective, irreversible EGFRi)

NSCLC

Trial	Population	Patients	Design	Endpoints	Status
Phase III **ADAURA** **NCT02511106**	Adjuvant EGFRm NSCLC	682	• Arm 1: *Tagrisso* QD following complete tumour resection, with or without chemo • Arm 2: placebo Global trial - 25 countries	• Primary endpoint: DFS • Secondary endpoints: DFS Rate, OS, OS Rate, QoL	• FPCD: Q4 2015 • LPCD: Q1 2019 • Data readout: Q2 2020 • Trial unblinded due to efficacy • DFS primary endpoint met
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	200	• Arm 1: *Tagrisso* • Arm 2: placebo Global trial - 17 countries	• Primary endpoint: PFS (BICR) • Secondary endpoints: CNS PFS, OS, DoR, ORR, DCR	• FPCD: Q4 2018 • Data anticipated: 2022+
Phase III **ASTRIS** **NCT02474355**	Real world setting in adult patients with advanced or metastatic, EGFRm T790M+ NSCLC	3020	• Single-arm trial - *Tagrisso* Global trial - 16 countries	• Primary endpoints: OS and safety • Secondary endpoint: PFS	• FPCD: Q3 2015 • LPCD: Q4 2017
Phase II **ELIOS** **NCT03239340**	EGFR TKI treatment-naïve patients with locally-advanced or metastatic EGFRm NSCLC	150	• Single arm trial - *Tagrisso* Global trial - five countries	• Primary endpoint: proportion of patients with a given tumour genetic and proteomic marker at the point of disease progression as defined by the investigator • Secondary endpoints: PFS, ORR, DoR	• FPCD: Q2 2018
Phase I **ODIN-BM** **NCT03463525**	Patients with EGFRm NSCLC with brain metastases	8	• Single-arm trial - *Tagrisso*	• Primary endpoints: assessments of brain standard uptake value (SUV) and pharmacokinetics (PK) • Secondary endpoint: PK	• FPCD: Q4 2018 • LPCD: Q1 2020 • Data anticipated: Q4 2021

Tagrisso (highly-selective, irreversible EGFRi)

NSCLC, combinations

Trial	Population	Patients	Design	Endpoints	Status
Phase III **NeoADAURA** **NCT04351555**	Neoadjuvant EGFRm NSCLC	351	• Arm 1: placebo plus pemetrexed/carboplatin or pemetrexed/cisplatin • Arm 2: *Tagrisso* plus pemetrexed/carboplatin or pemetrexed/cisplatin • Arm 3: *Tagrisso* Global trial – 23 countries	• Primary endpoint: mPR • Secondary endpoints: cPR, EFS, DFS, OS	• FPCD Q1 2021 • Data anticipated: 2022+
Phase III **FLAURA2** **NCT04035486**	1st-line EGFRm NSCLC	586	• Arm 1: *Tagrisso* plus 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, PK	• FPCD: Q4 2019 • Data anticipated: 2022+
Phase II **SAVANNAH** **NCT03778229**	EGFRm / MET+, locally advanced or metastatic NSCLC who have progressed following treatment with *Tagrisso*	259	• Single arm trial: *Tagrisso* + *Orpathys* Global trial	• Primary endpoint: ORR • Secondary endpoints include PFS, DoR and OS	• FPCD Q1 2019 • Data anticipated: 2022+
Phase II **ORCHARD** **NCT03944772**	Advanced EGFRm NSCLC patients who have progressed on first line *Tagrisso* treatment	182	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) • No intervention: observational cohort Global trial - 8 countries	• Primary endpoint: ORR • Secondary endpoints: PFS, DoR, OS, safety and tolerability	• FPCD: Q3 2019 • Data anticipated: 2022+
Phase II **COMPEL** **NCT04606771**	EGFRm/MET amplified advanced NSCLC	56	• *Tagrisso* and *Orpathys* contribution of components	• Primary endpoint: ORR • Secondary endpoints: PFS, DoR, OS	• FPCD: Q4 2020 • Data anticipated: H2 2022

Imfinzi (PD-L1 mAb)

NSCLC, early disease

Trial	Population	Patients	Design	Endpoints	Status
Phase III MERMAID-1 NCT04385368	Completely resected Stage II and III NSCLC	332	• Arm 1: *Imfinzi* + SoC chemo • Arm 2: placebo + SoC chemo	• Primary endpoint: DFS • Secondary endpoints: DFS, OS	• FPCD: Q3 2020 • Data anticipated: 2022+
Phase III MERMAID-2 NCT04642469	Completely resected Stage II-III NSCLC	284	• Arm 1: *Imfinzi* • Arm 2: placebo	• Primary endpoint: DFS • Secondary endpoints: DFS, PFS, OS	• FPCD: Q3 2021 • Data anticipated: 2022+
Phase III AEGEAN NCT03800134	Neoadjuvant NSCLC patients Stage II and III resected NSCLC (incl. EGFR/ALK positive)	800	• Arm 1: *Imfinzi* + platinum-based chemo • Arm 2: placebo + platinum-based chemo	• Primary endpoints: pCR, EFS • Secondary endpoint: mPR	• FPCD: Q1 2019 • Data anticipated: 2022+
Phase III ADJUVANT BR.31 NCT02273375 Partnered	Adjuvant NSCLC patients Ib (≥4cm) – stage IIIa resected NSCLC (incl. EGFR/ALK positive)	1360	• Arm 1: *Imfinzi* mg/kg i.v. Q4W x 12m • Arm 2: placebo Global trial	• Primary endpoint: DFS • Secondary endpoint: OS	• FPCD: Q1 2015 • LPCD: Q1 2020 • Data anticipated: 2022+
Phase III PACIFIC-2 NCT03519971	Unresected, locally-advanced NSCLC	300	• Arm 1: *Imfinzi* i.v. Q4W + chemo/RT • Arm 2: placebo + chemo/RT ex US global trial	• Primary endpoint: PFS • Secondary endpoints: OS, ORR	• FPCD: Q2 2018 • LPCD: Q3 2019 • Data anticipated: H1 2022
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: 2022+
Phase III PACIFIC-5 NCT03706690	Unresected, locally-advanced NSCLC	360	• Arm 1: *Imfinzi* i.v. Q4W following chemo/RT • Arm 2: placebo following chemo/RT ex US global trial, China focus	• Primary endpoint: PFS • Secondary endpoint: OS	• FPCD: Q1 2019 • Data anticipated: 2022+

Imfinzi (PD-L1 mAb)

Lung cancer, early disease

Trial	Population	Patients	Design	Endpoints	Status
Phase II HUDSON NCT03334617	NSCLC, patients who progressed on an anti-PD-1/PD-L1 containing therapy	340	Open-label, biomarker-directed, multicentre trial • Module 1: *Imfinzi* and *Lynparza* • Module 2: *Imfinzi* and danvatirsen • Module 3: *Imfinzi* and ceralasertib (AZD6738) • Module 4: *Imfinzi* and vistusertib • Module 5: *Imfinzi* and oleclumab • Module 6: *Imfinzi* and *Enhertu* • Module 7: *Imfinzi* and cediranib • Module 8: ceralasertib	• Primary outcome: ORR • Secondary outcomes: efficacy including OS, PFS, DCR, and safety and tolerability, DoR	• FPCD: Q1 2018 • Data anticipated: 2022+
Phase II COAST NCT03822351	Stage III NSCLC unresectable	189	• Arm A: *Imfinzi* • Arm B: *Imfinzi* + oleclumab • Arm C: *Imfinzi* + monalizumab	• Primary endpoint: OR per RECIST v1.1	• FPCD: Q4 2018 • Data readout: Q3 2021
Phase II NeoCOAST NCT03794544	Resectable, early-stage NSCLC	84	• Arm A: *Imfinzi* • Arm B: *Imfinzi* + oleclumab • Arm C: *Imfinzi* + monalizumab • Arm D: *Imfinzi* + danvatirsen	• Primary endpoint: Major pathological response rate	• FPCD: Q1 2019 • Data anticipated: Q4 2021
Phase I/II SCope-D1	NSCLC SCLC	124	Open-label, multicentre trial to evaluate the safety, pharmacokinetics, and preliminary efficacy of subcutaneous *Imfinzi*	• Primary endpoints: PK, safety	• FPCD: Q4 2021 • Data anticipated: 2022+

Imfinzi (PD-L1 mAb) +/- treme (CTLA-4 mAb)

Lung cancer, advanced disease

Trial	Population	Patients	Design	Endpoints	Status
Phase III PEARL NCT03003962	NSCLC 1L	650	• Arm 1: *Imfinzi* Q4W • Arm 2: chemotherapy Asia trial	• Primary endpoint: OS	• FPCD: Q1 2017 • LPCD: Q1 2019 • Data anticipated: H1 2022
Phase III POSEIDON NCT03164616	NSCLC 1L	1000	• Arm 1: *Imfinzi* + chemo • Arm 2: *Imfinzi* + tremelimumab + chemo • Arm 3: SoC	• Primary endpoints: OS, PFS	• FPCD: Q2 2017 • LPCD: Q4 2018 • Data readout: Q4 2019 • PFS primary endpoint met • OS data readout: Q2 2021
Phase II/III Lung Master Protocol NCT02154490 Partnered	Stage IV squamous NSCLC patients Biomarker-targeted 2L therapy	140	• Subtrial A: *Imfinzi* (non-match for other biomarker driven subtrials) i.v. Q2W single arm *Imfinzi* Phase II only • Subtrial B: PI3K inhibitor vs. docetaxel • Subtrial C: CDK4/6 inhibitor vs. docetaxel • Subtrial D: AZD4547 (FGFR inhibitor) vs. docetaxel • Subtrial E: cMET/HGFR Inhibitor + erlotinib vs. erlotinib	• Primary endpoints: ORR, PFS, OS	• FPCD: Q2 2014 • Data anticipated: 2022+
Phase II MAGELLAN NCT03819465	NSCLC 1L	212	• Arm A1: *Imfinzi* • Arm A2: *Imfinzi* + danvatirsen • Arm A3: *Imfinzi* + oleclumab • Arm A3: MEDI5752 • Arm B1: *Imfinzi* + Investigator's choice of chemo • Arm B2: *Imfinzi* + danvatirsen + Investigator's choice of chemo • Arm B3: *Imfinzi* + oleclumab + Investigator's choice of chemo • Arm B4: MEDI5752	• Primary endpoints: safety & tolerability • Secondary endpoints: ORR, DoR, PFS, OS, PK, ADA	• FPCD: Q1 2019 • Data anticipated: 2022+
Phase Ib Study 006 NCT02000947	NSCLC (IO naïve and IO pretreated patient cohorts)	459	Dose escalation: • Minimum 5 cohorts exploring various treme Q4W and *Imfinzi* i.v. Q4W dose combinations, higher dose levels and alternate Q2 schedule added with amendment Dose expansion: • MTD for the combination in escalation to be explored in expansion North American, EU and ROW trial centres	• Primary endpoints: safety, Optimal biologic dose for the combination, OR • Secondary endpoints include: antitumour activity, PK and immunogenicity	• FPCD: Q4 2013 • LPCD: Q4 2016 • Data readout: Q3 2020

Imfinzi (PD-L1 mAb) +/- treme (CTLA-4 mAb)

SCLC, advanced disease

Trial	Population	Patients	Design	Endpoints	Status
Phase III ADRIATIC NCT03703297	Limited stage SCLC 1L following platinum-based concurrent chemoradiation therapy	600	• Arm 1: *Imfinzi* + tremelimumab (4 doses) • Arm 2: *Imfinzi* • Arm 3: placebo	• Primary endpoints: PFS, OS	• FPCD: Q4 2018 • Data anticipated: H2 2022

Imfinzi (PD-L1 mAb)

Other cancers

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: 2022+
Phase III NIAGARA NCT03732677	Muscle-invasive bladder cancer	1064	• Arm 1: *Imfinzi* in combination with gemcitabine + cisplatin, *Imfinzi* maintenance • Arm 2: gemcitabine + cisplatin	• Coprimary endpoints: pCR , EFS	• FPCD: Q4 2018 • LPCD: Q3 2021 • Data anticipated: 2022+
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 for Arm 1 vs Arm 3 • Secondary endpoint: PFS for Arm 2 vs Arm 3 , OS	• FPCD: Q1 2019 • LPCD: Q3 2021 • Data anticipated: H2 2022
Phase III EMERALD-2 NCT03847428	Adjuvant therapy in HCC	888	• Arm 1: *Imfinzi* + bevacizumab • Arm 2: *Imfinzi* + placebo • Arm 3: placebo + placebo	• Primary endpoint: PFS for Arm 1 vs Arm 2 • Secondary endpoints: PFS Arm 2 vs Arm 3, OS, RFS at 24m	• FPCD: Q2 2019 • Data anticipated: 2022+
Phase III KUNLUN NCT04550260	Locally advanced, unresectable ESCC	600	• Arm 1: *Imfinzi* + definitive CRT • Arm 2: placebo + definitive CRT	• Primary endpoint: PFS • Secondary endpoint: OS	• FPCD: Q4 2020 • Data anticipated: 2022+
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, pCR Arm 1 vs Arm 2	• FPCD: Q4 2020 • Data anticipated: 2022+
Phase III SAMETA NCT05043090	MET-Driven, unresectable and locally advanced or metastatic papillary renal cell carcinoma	200	• *Orpathys* + *Imfinzi* versus sunitinib and *Imfinzi* monotherapy	• Primary endpoint: PFS • Secondary endpoints include OS, ORR, DoR and DCR	• FPCD: Q4 2021 • Data anticipated: 2022+

**Phase Ib/II
COLUMBIA 1
NCT04068610**
	1L metastatic MSS-CRC	112	• Part 1 S1 FOLFOX + bevacizumab + *Imfinzi* + oleclumab
			• Part 2 Control 1 FOLFOX + bevacizumab
			• Part 2 E1 FOLFOX + bevacizumab + *Imfinzi* + oleclumab
				• Primary endpoints:
				  • Part 1: safety
				  • Part 2: efficacy (OR)
				• Secondary endpoints:
				  • Part 1: efficacy (OR), BOR, DoR, PFS
				  • Part 2: safety and efficacy (BOR, DoR, DC, PFS, OS)
					• FPCD: Q3 2019
					• Data anticipated: 2022+

Imfinzi (PD-L1 mAb) +/- treme (CTLA-4 mAb)

Other cancers, advanced disease

Trial	Population	Patients	Design	Endpoints	Status
Phase III NILE NCT03682068	Bladder cancer 1L	1220.	• Arm 1: *Imfinzi* + tremelimumab + SoC • Arm 2: *Imfinzi* + SoC • Arm 3: SoC	• Primary endpoint: OS	• FPCD: Q4 2018 • LPCD: Q2 2021 • Data anticipated: 2022+
Phase III HIMALAYA NCT03298451	HCC 1L	1324	• Arm 1: *Imfinzi* + tremelimumab • Arm 2: *Imfinzi* • Arm 3: sorafenib	• Primary endpoint: OS • Secondary endpoints: PFS, TTP, ORR	• FPCD: Q4 2017 • LPCD: Q4 2019 • Data readout: Q4 2021 • Primary endpoint met
Phase III TOPAZ-1 NCT03875235	BTC 1L	757	• Arm 1: *Imfinzi* + gemcitabine + cisplatin • Arm 2: placebo + gemcitabine + cisplatin Global trial	• Primary endpoint: OS • Secondary endpoints: PFS, ORR, DoR	• FPCD Q2 2019 • LPCD: Q4 2020 • Data readout: Q4 2021 • Primary endpoint met
Phase III CALLA NCT03830866	Locally advanced cervical cancer	770	• Arm 1 Imfinzi + EBRT + brachytherapy with platinum • Arm 2 placebo + EBRT + brachytherapy with platinum Global trial	• Primary endpoint: PFS • Secondary endpoints: OS, CR rate, DoR, ORR, safety/tolerability	• FPCD: Q1 2019 • LPCD: Q4 2020 • Data anticipated: H1 2022
Phase III VOLGA NCT04960709	Muscle invasive bladder cancer ineligible to cisplatin	830	• Arm 1: *Imfinzi* + tremelimumab + enfortumab vedotin • Arm 2: *Imfinzi* + enfortumab vedotin • Arm 3: SoC cystectomy	• Primary endpoints: safety, EFS, pCR • Secondary endpoints: OS	• FPCD: Q4 2021 • Data anticipated: 2022+
Phase Ib/II Study 22 NCT02519348	Hepatocellular carcinoma	545	• Arm A: *Imfinzi* + tremelimumab • Arm B: *Imfinzi* 2L • Arm C: tremelimumab 2L • Arm D: Imfinzi + tremelimumab • Arm E: *Imfinzi* in combination with bevacizumab	• Primary endpoints: safety & tolerability, DLTs • Secondary endpoints: ORR, DoR, OS	• FPCD: Q4 2015 • Data readout: Q2 2020

Imfinzi (PD-L1 mAb) +/- treme (CTLA-4 mAb)

Other cancers, advanced disease

Trial	Population	Patients	Design	Endpoints	Status
Phase III **STRONG** **NCT03084471**	Advanced solid malignancies	1200	• Arm 1: *Imfinzi* • Arm 2: *Imfinzi* + tremelimumab	• Primary endpoint: safety	• FPCD: Q2 2017 • Data anticipated: 2022+
Phase II **BEGONIA** **NCT03742102**	mTNBC 1L	220	• Arm 1 *Imfinzi* + paclitaxel • Arm 2 *Imfinzi* + paclitaxel + capivasertib • Arm 5 *Imfinzi* + paclitaxel + oleclumab • Arm 6 *Imfinzi* + *Enhertu* • Arm 7 *Imfinzi* + datopotamab deruxtecan Global trial	• Primary endpoints: safety and tolerability • Secondary endpoints: ORR, PFS, DoR, OS, PK, ADA	• FPCD: Q1 2019 • Data anticipated: 2022+
Phase I/II **Study 1108** **NCT01693562**	Solid tumours	1022	Dose escalation: 5 cohorts at Q2W and 1 cohort at Q3W Dose expansion: 16 tumour type cohorts at the Q2W MTD defined during dose escalation Dose exploration: cohort at 20mg Q4W Global trial - nine countries	• Primary endpoints: safety, optimal biologic dose • Secondary endpoints include: PK, immunogenicity and antitumour activity	• FPCD: Q3 2012 • LPCD: Q4 2016 • Data readout: Q2 2020
Phase I **CLOVER** **NCT03509012**	HNSCC, NSCLC, SCLC	167	*Imfinzi* +/- treme in combination with chemoradiation in advanced solid tumours	• Primary endpoint: safety	• FPCD: Q2 2018 • Dara readout: Q4 2021

Lynparza (PARP inhibitor)

Multiple cancers

Trial	Population	Patients	Design	Endpoints	Status
Phase III **OlympiA** **NCT02032823** **Partnered**	BRCAm adjuvant breast cancer	1836	• Arm 1: *Lynparza* BID 12-month duration • Arm 2: placebo 12-month duration Global trial partnership with Breast International Group and National Cancer Institute/NRG Oncology	• Primary endpoint: invasive disease-free survival (iDFS) • Secondary endpoint: 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* • Arm 2: placebo Global trial, 12 countries	• Primary endpoints: PFS (BRCAwt HRD+ve), PFS (BRCAwt) • Secondary endpoints: OS, TFST, PFS2	• FPCD: Q3 2021 • Data anticipated: 2022+

Lynparza (PARP inhibitor)

Other combinations

Trial	Population	Patients	Design	Endpoints	Status
Phase III **PROpel** **NCT03732820**	Metastatic castration-resistant prostate cancer 1L	904	• Arm 1: *Lynparza* + abiraterone • Arm 2: placebo + abiraterone Global trial, including China cohort	• Primary endpoint: rPFS • Secondary endpoints: TFST, TTPP, OS	• FPCD: Q4 2018 • Data readout: Q3 2021 • Primary endpoint met
Phase III **LYNK-003** **NCT04456699** **Partnered**	Advanced colorectal cancer 1L maintenance	525	• Arm 1: bevacizumab + 5-FU maintenance • Arm 2: bevacizumab + *Lynparza* maintenance • Arm 3: *Lynparza* maintenance Global trial	• Primary endpoint: PFS • Secondary endpoints: OS, ORR, DoR, AEs	• FPCD: Q3 2020 • Data anticipated: 2022+
Phase II/III **GY005** **NCT02502266** **Externally sponsored**	Recurrent platinum resistant/refractory ovarian cancer	680	• Arm 1: chemo • Arm 2: cediranib + *Lynparza* • Arm 3: cediranib • Arm 4: *Lynparza* US, Canada	• Primary endpoints: PFS, OS • Secondary endpoints: ORR, QoL, safety	• FPCD: Q2 2016 • Data anticipated: 2022+
Phase II **LYNK-002** **NCT03742895** **Partnered**	HRRm or HRD-positive advanced cancer	390	• Arm 1: *Lynparza* Global trial	• Primary endpoints: ORR • Secondary endpoints: DOR, OS, PFS, AE, Prog by CA-125	• FPCD: Q1 2019

Lynparza (PARP inhibitor)

Imfinzi combinations

Trial	Population	Patients	Design	Endpoints	Status
Phase III **DuO-O** **NCT03737643**	Advanced ovarian cancer 1L	1,256	Non tBRCAm (tumour BRCA) patients • Arm 1: bevacizumab • Arm 2: bevacizumab + *Imfinzi* • Arm 3: bevacizumab + *Imfinzi* + *Lynparza* tBRCAm patients • bevacizumab (optional) + *Imfinzi* + *Lynparza* Global trial	• Primary endpoint: PFS • Secondary endpoints: OS, PFS2	• FPCD: Q1 2019 • Data anticipated: 2022+
Phase III **DuO-E** **NCT04269200**	Advanced and recurrent endometrial cancer 1L	699	• Arm 1: chemo + *Imfinzi* placebo followed by *Imfinzi* placebo and *Lynparza* placebo • Arm 2: chemo + *Imfinzi* followed by *Imfinzi* + *Lynparza* placebo • Arm 3: chemo + *Imfinzi* followed by *Imfinzi* + *Lynparza* Global Trial	• Primary endpoint PFS • Secondary endpoints: OS, PFS2, ORR, DoR	• FPCD: Q2 2020 • Data anticipated: 2022+
Phase II **ORION** **NCT03775486**	Stage IV NSCLC whose disease has not progressed following SoC chemo + *Imfinzi* maintenance therapy 1L	250	• Arm 1: *Imfinzi* + *Lynparza* • Arm 2: *Imfinzi* + placebo Global trial	• Primary endpoint: PFS • Secondary endpoints: OS, ORR, DoR, PFS in HRRm, PK, ADA	• FPCD Q1 2019 • Data readout: Q2 2021
Phase II **BAYOU** **NCT03459846**	Platinum-ineligible unresectable Stage IV urothelial cancer	154	• Arm 1: *Imfinzi* + *Lynparza* • Arm 2: *Imfinzi* + placebo Global trial	• Primary endpoint: PFS • Secondary endpoints: OS, DoR, ORR, PFS in HRRm, PFS6, PK, ADA, PRO	• FPCD: Q2 2018 • LPCD: Q3 2019 • Data readout: Q2 2021
Phase I / II **MEDIOLA** **NCT02734004**	gBRCAm ovarian cancer 2L+ gBRCAm HER2-negative breast cancer 1-3L SCLC 2L+ Gastric cancer 2L+	148	Dose until progression • Arm 1: *Lynparza* + *Imfinzi* Global trial	• Primary endpoints: DCR at 12 weeks, safety and tolerability	• FPCD: Q2 2016 • LPCD: Q2 2017
Phase I / II **MEDIOLA** **(Ovarian expansion)** **NCT02734004**	gBRCAm ovarian cancer 2L+ Non-gBRCAm ovarian cancer 2L+ Non-gBRCAm ovarian cancer 2L+	115	Dose until progression • Arm 1: *Lynparza* + *Imfinzi* • Arm 2: *Lynparza* + *Imfinzi* • Arm 3: *Lynparza* + *Imfinzi* + bevacizumab Global trial	• Primary endpoints: DCR at 12 weeks, ORR, safety and tolerability	• FPCD: Q2 2018 • LPCD: Q2 2020

Enhertu (trastuzumab deruxtecan, HER2 ADC)

Breast cancer

Trial	Population	Patients	Design	Endpoints	Status
Phase III **DESTINY-Breast02** **NCT03523585**	HER2-positive, unresectable and/or metastatic breast cancer pretreated with prior standard of care HER2 therapies, including trastuzumab emtansine	600	Randomised open label parallel assignment • *Enhertu* • Physician's choice of lapatinib + capecitabine or trastuzumab + capecitabine	• Primacy endpoint: PFS • Secondary endpoints: OS, ORR, DoR, CBR	• FPCD: Q3 2018 • LPCD: Q4 2020 • Data anticipated: H2 2022
Phase III **DESTINY-Breast03** **NCT03529110**	HER2-positive, unresectable and/or metastatic breast cancer previously treated with trastuzumab and taxane	500	Randomised open label parallel assignment • *Enhertu* • Ado-trastuzumab emtansine	• Primary endpoint: PFS • Secondary endpoints: OS, ORR, DoR, CBR	• FPCD: Q3 2018 • LPCD: Q2 2020 • Data readout: Q3 2021 • Primary endpoint met
Phase III **DESTINY-Breast04** **NCT03734029**	HER2-low, unresectable and/or metastatic breast cancer patients	540	Randomised open label parallel assignment • *Enhertu* • Physician's choice of SoC chemo (choice of capecitabine, eribulin, gemcitabine, paclitaxel or nab-paclitaxel)	• Primary end point: PFS • Secondary endpoints: OS, DoR, ORR	• FPCD: Q4 2018 • LPCD: Q4 2020 • Data anticipated: H1 2022
Phase III **DESTINY-Breast05** **NCT04622319**	High-risk HER2-positive patients with residual invasive breast cancer following neoadjuvant therapy	1600	Randomised open label parallel assignment • *Enhertu* • Ado-trastuzumab emtansine	• Primary endpoint: IDFS • Secondary endpoints: DFS, OS, DRFI, BMFI	• FPCD: Q4 2020 • Data anticipated: 2022+
Phase III **DESTINY-Breast06** **NCT04494425**	HER2-Low, HR+ breast cancer patients whose disease has progressed on endocrine therapy in the metastatic setting	850	Randomised open label parallel assignment • *Enhertu* • Investigator's choice standard of care chemotherapy (capecitabine, paclitaxel, nab-paclitaxel)	• Primary endpoint: PFS • Secondary endpoints: OS, DoR, ORR	• FPCD: Q3 2020 • Data anticipated: 2022+
Phase III **DESTINY-Breast09** **NCT04784715**	HER2-positive, metastatic breast cancer, no prior therapy for advanced or metastatic disease	1134	Randomised, parallel assignment • *Enhertu* + placebo • *Enhertu* + pertuzumab • Standard of care	• Primary endpoint: PFS • Secondary endpoints: OS, DoR, ORR	• FPCD: Q2 2021 • Data anticipated: 2022+

Enhertu (trastuzumab deruxtecan, HER2 ADC)

Breast cancer

Trial	Population	Patients	Design	Endpoints	Status
Phase Ib/II **DESTINY-Breast07** **NCT04538742**	HER2-positive metastatic breast cancer	350	Randomised open label sequential assignment • *Enhertu* • *Enhertu* + *Imfinzi* • *Enhertu* + pertuzumab • *Enhertu* + paclitaxel • *Enhertu* + *Imfinzi* + paclitaxel • *Enhertu* + tucatinib	• Primary endpoints: AE, SAE • Secondary endpoints: ORR, PFS, DoR, OS	• FPCD: Q1 2021 • Data anticipated: 2022+
Phase Ib **DESTINY-Breast08** **NCT04556773**	HER2-low metastatic breast cancer	185	Non-randomised open label parallel assignment • *Enhertu* + capecitabine • *Enhertu* + *Imfinzi* + paclitaxel • *Enhertu* + capivasertib • *Enhertu* + anastrozole • *Enhertu* + *Faslodex*	• Primary endpoints: AE, SAE • Secondary endpoints: ORR, PFS, DoR, OS	• FPCD: Q1 2021 • Data anticipated: 2022+

Enhertu (trastuzumab deruxtecan, HER2 ADC)

Gastric cancer

Trial	Population	Patients	Design	Endpoints	Status
Phase III **DESTINY-Gastric04** **NCT04704934**	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 • *Enhertu* • SoC chemo	• Primary endpoint: OS • Secondary endpoints: ORR, DoR, PFS, DcR, safety	• FPCD: Q2 2021 • Data anticipated: 2022+
Phase II **DESTINY-Gastric01** **NCT03329690**	HER2-overexpressing advanced gastric or gastroeosophogeal junction adenocarcinoma patients who have progressed on two prior treatment regimens	233	Randomised open label parallel assignment • *Enhertu* • SoC chemo • Two additional open label patient cohorts with lower levels of HER2 expression Japan and Korea	• Primary endpoint: ORR • Secondary endpoints: PFS, OS, DoR, DCR, TTF, range of PK endpoints	• FPCD: Q4 2017 • LPCD: Q2 2019 • Data readout: Q1 2020 • Primary endpoint met
Phase II **DESTINY-Gastric02** **NCT04014075**	HER2-positive gastric cancer that cannot be surgically removed or has spread	79	Open label single group assignment • *Enhertu* Western population	• Primary endpoint: ORR • Secondary endpoints: PFS, ORR, OS, DoR	• FPCD: Q4 2019 • LPCD: Q4 2020 • Data readout: Q2 2021
Phase Ib/II **DESTINY-Gastric03** **NCT04379596**	HER2-overexpressing gastric or gastroeosophogeal junction cancer patients	255	Open label parallel assignment Part 1: To determine recommended Phase II combination dose • 5 Arms combine *Enhertu* with standard of care chemotherapies (5-FU, capecitabine, oxaliplatin) and / or durvalumab Part 2: To assess efficacy of the selected combinations • Arm 2A: Standard chemotherapy (control) • Arm 2B: *Enhertu* monotherapy • Arm 2C: *Enhertu* with chemotherapy • Arm 2D: *Enhertu* with chemotherapy and pembrolizumab • Arm 2E: *Enhertu* and pembrolizumab	• Part 1 Primary endpoint: safety • Part 2 Primary endpoint: ORR • Secondary endpoints: DoR, DCR, PFS, OS, range of PK endpoints, ADAs	• FPCD: Q2 2020
Phase II **DESTINY-Gastric06** **NCT04989816**	HER2-positive gastric cancer or gastro-esophageal junction adenocarcinoma patients who have progressed on two prior treatment regimens	75	Open label single group assignment • *Enhertu* China	• Primary endpoint: ORR • Secondary endpoints: PFS, ORR, DCR, OS, DoR, safety	• FPCD: Q2 2021

Enhertu (trastuzumab deruxtecan, HER2 ADC)

Other cancers

Trial	Population	Patients	Design	Endpoints	Status
Phase III **DESTINY-Lung04** **NCT05048797**	HER2 mutated, unresectable, locally advanced/metastatic NSCLC	264	Randomised parallel group assignment • Arm 1: *Enhertu* • Arm 2*:* cisplatin or carboplatin	• Primary endpoint: PFS • Secondary endpoints: OS, ORR, DoR, safety	• Initiating
Phase II **DESTINY-Lung01** **NCT03505710**	HER2-over-expressing or mutated, unresectable and/or metastatic NSCLC	170	Non randomised parallel group assignment • *Enhertu*	• Primary endpoint: ORR • Secondary endpoints: DoR, PFS, OS	• FPCD: Q2 2018 • Data readout: Q3 2021 • Positive primary results
Phase II **DESTINY-Lung02** **NCT04644237**	HER2-mutated, unresectable and/or metastatic NSCLC	150	Randomised parallel group assignment • Arm 1: *Enhertu* 6.4 mg/kg • Arm 2*: Enhertu* 5.4mg/kg	• Primary endpoint: ORR • Secondary endpoints: DoR, DCR, PFS, OS, PK	• FPCD: Q1 2021 • Data anticipated 2022+
Phase Ib **DESTINY-Lung03** **NCT04686305**	HER2-over-expressing, unresectable and/or metastatic NSCLC	136	Non randomised parallel group assignment Part 1: To determine recommended combination dose • 3 Arms combine *Enhertu* with standard of care chemotherapies (cisplatin, carboplatin or pemetrexed) and *Imfinzi. Arm 1D: Enhertu monotherapy arm* Part 2: To assess efficacy of the selected combinations • *Arm 1: Enhertu* + cisplatin *+ Imfinzi* • *Arm 2: Enhertu* + carboplatin *+ Imfinzi* • *Arm 3: Enhertu* + pemetrexed *+ Imfinzi* • Arm 4: *Enhertu* + *Imfinzi*	• Primary endpoint: safety • Secondary endpoints: ORR, DoR, DCR, PFS, OS, range of PK endpoints	• FPCD: Q4 2021

Enhertu (trastuzumab deruxtecan, HER2 ADC)

Other cancers

Trial	Population	Patients	Design	Endpoints	Status
Phase II **DESTINY-PanTumour02** **NCT04482309**	HER2 expressing tumours	280	Non randomised single group assignment • *Enhertu*	• Primary endpoint: ORR • Secondary endpoints: DoR, DCR, PFS, OS	• FPCD: Q4 2020
Phase II **DESTINY-PanTumour01** **NCT04639219**	HER2 mutant tumours	100	Non-randomised single group assignment • *Enhertu*	• Primary endpoint: ORR • Secondary endpoints: DoR, DCR, PFS, PK	• FPCD: Q1 2021
Phase II **DESTINY-CRC02** **NCT04744831**	HER2 overexpressing advanced or metastatic colorectal cancer	120	Randomised parallel group assignment • Arm 1: *Enhertu* 6.4 mg/kg • Arm 2: *Enhertu* 5.4mg/kg	• Primary endpoint: ORR • Secondary endpoint: PFS, OS, DoR, DCR, range of PK endpoints	• FPCD: Q1 2021
Phase Ib **U106** **NCT04042701**	HER2 expressing locally advanced/metastatic breast or NSCLC	115	Non randomised parallel group assignment • *Enhertu* + pembrolizumab Global trial 2 countries	• Primary endpoints: DLT, ORR • Secondary endpoints: DoR, DCR, PFS, TTR, OS	• FPCD: Q2 2020
Phase Ib **U105** **NCT03523572**	HER2-expressing breast and urothelial cancer	99	Non randomised sequential assignment • *Enhertu* + nivolumab Global trial 7 countries	• Primary endpoints: DLT, ORR, TEAEs • Secondary endpoints: DoR, DCR, PFS, TTR, OS, ORR (investigator)	• FPCD: Q3 2018 • Data readout: Q3 2021

Calquence (BTK inhibitor)

Blood cancers

Trial	Population	Patients	Design	Endpoint(s)	Status
Phase III **ACE-CL-007 (ELEVATE-TN)** **NCT02475681**	Previously untreated CLL	535	• Arm A: chlorambucil + obinutuzumab • Arm B: *Calquence* + obinutuzumab • Arm C: *Calquence*	• Primary endpoint: PFS (Arm A vs. Arm B) • Secondary endpoints: IRC (independent review committee) assessed ORR, OS (Arm A vs. Arm B vs. Arm C)	• FPCD: Q2 2015 • Data readout: Q2 2019 • Primary endpoint met
Phase III **ACE-CL-311** **NCT03836261**	Previously untreated CLL fit	780	• Arm A; *Calquence* + venetoclax • Arm B: *Calquence* + venetoclax + obinutuzumab • Arm C: FCR or BR	• Primary endpoint: IRC PFS (A vs C) • Secondary endpoint: IRC PFS (B vs C); INV PFS (A vs C; B vs C)	• FPCD: Q1 2019 • Data anticipated: 2022+
Phase III **ACE-CL-309 (ASCEND)** **NCT02970318**	Relapsed/refractory CLL	306	• Arm A: *Calquence* • Arm B: rituximab + idelalisib or bendamustine (investigator's choice)	• Primary endpoint: IRC assessed PFS (Arm A vs. Arm B) • Secondary endpoints: INV-assessed ORR, OS, DoR, PROs	• FPCD Q3 2016 • Data readout: Q2 2019 • Primary endpoint met
Phase III **ACE-CL-006 (ELEVATE-RR)** **NCT02477696**	Relapsed/refractory high risk CLL	533	• Arm A: *Calquence* • Arm B: ibrutinib	• Primary endpoint: PFS • Secondary endpoints: comparison of incidence of infections, RTs (Richter's Transformation) and atrial fibrillation, OS	• FPCD: Q2 2015 • Data readout: Q1 2021 • Primary endpoint met
Phase III **ACE-LY-308 (ECHO)** **NCT02972840**	Previously untreated MCL	546	• Arm A: *Calquence* + bendamustine + rituximab • Arm B: bendamustine + rituximab	• Primary endpoint: PFS by Lugano Classification for NHL • Secondary endpoints: IA, PFS, ORR, DoR, time to response, OS	• FPCD: Q1 2017 • Data anticipated: H2 2022
Phase III **ESCALADE** **NCT04529772**	DLBCL	600	*Calquence* + rituximab, cyclophosphamide, doxorubicin, vincristine, and prednisone	• Primary endpoints: safety, ORR	• FPCD: Q2 2020 • Data anticipated: 2022+
Phase II **15-H-0016** **NCT02337829**	Relapsed/refractory and treatment naïve/del17p CLL/SLL	48	*Calquence* monotherapy • Arm A: lymph node biopsy • Arm B: bone marrow biopsy	• Primary endpoint: ORR	• FPCD: Q4 2014 • Data anticipated: 2022+

Calquence (BTK inhibitor)

Blood cancers

Trial	Population	Patients	Design	Endpoint(s)	Status
Phase I/II **ACE-CL-001** **NCT02029443**	CLL/SLL/Richter's transformation	306	*Calquence* monotherapy Dose escalation and expansion	• Primary endpoints: safety, PK, PD	• FPCD: Q1 2014 • Data anticipated: Q4 2021
Phase Ib **ACE-LY-106** **NCT02717624**	MCL	70	*Calquence* in combination with bendamustine and rituxumab • Arm A: treatment naive • Arm B: relapsed/refractory • Arm C: treatment naïve: *Calquence* + venetoclax + rituxumab	• Primary endpoint: safety	• FPCD: Q1 2016 • Data anticipated: 2022+
Phase I **ACE-LY-003** **NCT02180711**	Relapsed/refractory follicular lymphoma	80	• Arm A: *Calquence* • Arm B: *Calquence* + rituximab • Arm C: *Calquence* + rituximab + lenolidomide	• Primary endpoint: safety	• FPCD: Q1 2015 • Data anticipated: 2022+
Phase I **ACE-CL-003** **NCT02296918**	CLL/SLL/PLL	69	*Calquence* + obinutuzumab • Arm A: relapsed/refractory • Arm B: treatment naïve *Calquence* + venetoclax + rituxumab • Arm C: relapsed/refractory • Arm D: treatment naïve	• Primary endpoints: safety, ORR • Secondary endpoints: PD, PFS, TTNT, OS	• FPCD: Q4 2014 • Data anticipated: 2022+

Calquence (BTK inhibitor)

Blood and other cancers

Trial	Population	Patients	Design	Endpoint(s)	Status
Phase III **CL-312 (ASSURE)** **NCT04008706**	CLL TN and R/R	600	• Arm A: treatment naïve • Arm B: relapsed/refractory • Arm C: prior BTKi therapy • Arm D: concomitant vitamin K antagonists	• Primary endpoint: safety	• Data anticipated: 2022+
Phase I/II **D8220C0007** **NCT03932331**	Chinese adults R/R MCL and R/R CLL	105	Part 1: R/R B-cell Malignancies Part 2: Cohort A: R/R MCL Part 2: Cohort B: R/R CLL	• Primary endpoints: safety, ORR	• FPCD: Q2 2020 • Data anticipated: H1 2022
Phase I **NCT03198650**	Japanese adults with advanced B-cell malignancies	34	Dose confirmation and expansion • *Calquence* Dose confirmation • *Calquence* + obinutuzumab	• Primary endpoints: safety, PK	• FPCD: Q2 2017 • Data anticipated: H2 2022
Phase I **D8220C00018** **NCT04488016**	Healthy volunteers	28	• Part 1: Rel bioavailabilty for capsule vs. tablet • Part 2: Rel bioavailabilty for oral solution of tablet	• Primary endpoint: safety	• FPCD: Q2 2019 • Data readout: Q1 2021
Phase I **D8223C00013** **NCT04768985**	Healthy volunteers	66	• Arm A: *Calquence* tablet • Arm B: *Calquence* capsule	• Primary endpoint: bioequivalence	• FPCD: Q1 2021 • Data anticipated: Q4 2021

Koselugo (selumetinib, MEK inhibitor)

Paediatric neurofibromatosis type 1, solid tumours

Trial	Population	Patients	Design	Endpoints	Status
Phase II SPRINT NCT01362803 Partnered	Paediatric NF1	50 (stratum 1) 25 (Stratum 2)	Single arm: *Koselugo* 25mg/m^2 BID with 2 strata: • Stratum 1: PN related morbidity present at enrolment • Stratum 2: no PN related morbidity present at enrolment	• Primary endpoint: Complete partial and complete response rate measured by volumetric MRI, Duration of response and functional outcomes/QoL	• FPCD: Q3 2015 • LPCD: Q4 2016 • Data readout: Q1 2019 • Primary endpoint met
Phase I Japan PK / Safety trial NCT04495127 Partnered	Paediatric inoperable NF1-PN patients	9-12	Open-label trial • *Koselugo* in Japanese paediatric NF1-PN patients	• Primary endpoint: safety • Secondary endpoints: PK, anti-tumour effect	• FPCD: Q3 2020 • LPCD: Q4 2020
Phase I China PK / Safety / Efficacy trial NCT04590235	Pediatric (2-17 years old), adult NF1	32	Single arm trial with 3 phases; Dose confirmation phase (n=6 for 3 cycles), Expansion phase (24mths post LSD) Long term follow up (60mths post LSD)	• Primary endpoints: safety, tolerability and PK • Secondary endpoints: efficacy (ORR, DoR; TTR; PFS)	• FPCD: Q4 2020
Phase I *Koselugo* with a low-fat meal compared to fasted state NCT05101148	Adolescents aged ≥ 12 to < 18 years at trial entry with a clinical diagnosis of NF1 related PN.	20 to be enrolled (to achieve 16 evaluable participants completing T2)	Single-arm, multiple dose, sequential, two or three period trial • *Koselugo* 25mg/m^2 BID given with a low-fat meal versus the same dose given in a fasted state.	• Primary endpoints: PK (steady state systemic exposure), safety (especially GI toxicity)	• FPCD: Q3 2021

Orpathys (savolitinib, MET inhibitor)

NSCLC and other cancers

Trial	Population	Patients	Design	Endpoints	Status
Phase II **NCT02897479** **Partnered**	Lung PSC and other NSCLC	65	Single arm trial • *Orpathys* QD China	• Primary endpoint: ORR • Secondary endpoints: PFS, safety parameters	• FPCD: Q1 2017 • Data readout: Q2 2020
Phase I **NCT01985555** **Partnered**	Advanced NSCLC (all comers)	85	Dose escalation trial China	• Primary endpoints: safety and tolerability • Secondary endpoints: PK profile	• FPCD: Q2 2013 • Data readout: Q3 2019

Capivasertib (AKT inhibitor)

Breast cancer, 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: capivasertib + paclitaxel • Arm 2: placebo + paclitaxel	• Primary endpoint: OS	• FPCD: Q3 2019 • Data anticipated: 2022+
Phase III CAPItello-291 NCT04305496	2L and beyond in AI resistant locally advanced (Inoperable) or metastatic HR+/HER2− breast cancer	834	Double-blind randomised comparative trial • Arm 1: capivasertib + *Faslodex* • Arm 2: placebo + *Faslodex*	• Primary endpoint: PFS	• FPCD: Q2 2020 • Data anticipated: 2022+
Phase III CAPItello-281 NCT04493853	De novo PTEN deficient metastatic hormone sensitive prostate cancer	1000	Double-blind randomised comparative trial • Arm 1: capivasertib + abiraterone • Arm 2: placebo + abiraterone	• Primary endpoint: rPFS	• FPCD: Q3 2020 • Data anticipated: 2022+
Phase III CAPItello-292 NCT04862663	1L triplet in early relapse/endocrine-resistant locally advanced (inoperable) or metastatic HR+/HER2- breast cancer	700	Double-blind randomised comparative trial • Arm 1: capivasertib + palbociclib + *Faslodex* • Arm 2: placebo + palbociclib + *Faslodex*	• Primary endpoint: PFS	• FPCD Q2 2021 • Data anticipated 2022+

Monalizumab (NKG2a mAb)

Cancers

Trial	Population	Patients	Design	Endpoints	Status
Phase III **INTERLINK-1** **NCT04590963**	Recurrent or metastatic HNSCC, 2L	600	• Arm A: monalizumab + cetuximab i.v. • Arm B: placebo + cetuximab i.v. Global	• Primary: OS • Secondary: PFS, ORR, DoR	• FPCD: Q4 2020 • Data anticipated: 2022+
Phase I/II **NCT02671435**	Advanced solid tumours	381	Escalation phase • monalizumab + *Imfinzi* i.v. Expansion phase • monalizumab + *Imfinzi* i.v. recommended dose Exploration phase • monalizumab + *Imfinzi* i.v. recommended dose + SoC systemic therapy with or without biologic agent and monalizumab in combination with a biologic agent in adult subjects with CRC Global	• Primary endpoints: safety • Exploration phase primary endpoint: Objective Response per RECIST • Secondary endpoints include: tumour response (OR, DC, DoR, PFS and OS), immunogenicity, pharmacokinetics, pharmacodynamics	• FPCD: Q2 2016 • Data anticipated: 2022+

Camizestrant (AZD9833, next generation oral SERD)

Breast cancer

Trial	Population	Patients	Design	Endpoints	Status
Phase III **SERENA-4** **NCT04711252**	HR+ HER2- breast cancer	1342	Randomised, double-blind, comparative trial • Arm A: camizestrant (AZD9833) + palbociclib • Arm B: anastrazole + palbociclib	• Primary endpoint: PFS • Secondary endpoint: OS, PFS2	• FPCD: Q1 2021 • Data anticipated: 2022+
Phase III **SERENA-6** **NCT04964934**	HR+ HER2- breast cancer	300	Randomised, double-blind, comparator trial • Arm A: camizestrant (AZD9833) plus palbociclib or abemaciclib • Arm B: anastrazole or letrozole plus palbociclib or abemaciclib	• Primary endpoint: PFS • Secondary endpoint: OS, PFS2	• FPCD Q3 2021 • Data anticipated: 2022+
Phase II **SERENA-2** **NCT04214288**	HR+ breast cancer	236	Randomised, open-label, parallel-group, multicentre trial • camizestrant (AZD9833) vs. i.m. *Faslodex* in women with advanced breast cancer.	• Primary outcome: mPFS	• FPCD: Q2 2020 • Data anticipated: H2 2022
Phase II **SERENA-3** **NCT04588298**	HR+ breast cancer	84	Randomised, open-label, parallel-group, multicentre trial	• Primary outcome: change in ER expression between pre- and on-treatment tumour biopsies	• FPCD: Q4 2020
Phase I **NCT04541433**	HR+ breast cancer	18	Open-label • anti-tumour activity of camizestrant (AZD9833) in Japanese women with endocrine resistant HR+ HER2- breast cancer that is not amenable to treatment with curative intent.	• Primary outcomes: safety and tolerability • Secondary outcome: PK	• FPCD: Q4 2020
Phase I **SERENA-1** **NCT03616587**	HR+ breast cancer	304	Escalation phase - open label multicentre trial • camizestrant (AZD9833) • camizestrant (AZD9833) + palbociclib, everolimus, abemeciclib or capivasertib. Expansion phase - randomised expansion cohort(s) at potential therapeutic dose(s) • camizestrant (AZD9833) • camizestrant (AZD9833) + palbociclib, everolimus, abemeciclib or capivasertib.	• Primary outcomes: safety and tolerability • Secondary outcomes: PK, antitumour activity	• FPCD: Q4 2018
Phase I **NCT04546347**	Healthy volunteers	32	Randomised, open-label	• Primary outcome: relative bioavailability of different tablet formulations and the effect of food	• FPCD: Q3 2020 • LPCD: Q4 2020 • Data readout: Q3 2021
Phase I **NCT04818632**	HR+ HER2- breast cancer Chinese patients	42	Dose escalation • camizestrant (AZD9833) Dose expansion • camizestrant (AZD9833) • camizestrant (AZD9833) + palbociclib • camizestrant (AZD9833) + everolimus	• Primary outcomes: safety and tolerability, PK • Secondary outcome: antitumour activity	• FPCD: Q1 2021 • Data anticipated: 2022+

Datopotamab deruxtecan (TROP2 ADC)

NSCLC

Trial	Population	Patients	Design	Endpoints	Status
Phase III TROPION-Lung01 NCT04656652 Partnered	Previously treated advanced or metastatic NSCLC without actionable genomic alterations	590	Randomised, open label, parallel assignment • Arm 1: datopotamab deruxtecan • Arm 2: docetaxel Global	• Primary endpoints: PFS, OS • Secondary endpoints: ORR, DoR, TTR, DCR, PK, ADA	• FPCD: Q1 2021 • Data anticipated: 2022+
Phase III TROPION-Lung08 Partnered	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	• Primary endpoints: PFS, OS	• Initiating • Data anticipated: 2022+
Phase II TROPION-Lung05 NCT04484142 Partnered	Advanced or metastatic NSCLC with actionable genomic alterations and progressed on or after kinase inhibitor therapy and platinum-based chemotherapy	150	Single-arm, open label • datopotamab deruxtecan Global trial	• Primary endpoint: ORR • Secondary endpoints: DOR, PFS, OS, safety, PK, ADA	• FPCD: Q1 2021 • Data anticipated: 2022+
Phase I TROPION-Lung02 NCT04526691 Partnered	Advanced or metastatic NSCLC	120	Open label, two-part (dose escalation, dose expansion), sequential assignment • datopotamab deruxtecan + pembrolizumab +/- platinum chemotherapy US, Japan	• Primary endpoints: DLT, safety • Secondary endpoints: ORR, DOR, PFS, OS, PK, ADA	• FPCD: Q4 2020 • Data anticipated: 2022+
Phase I TROPION-Lung04 NCT04612751 Partnered	Advanced or metastatic NSCLC	120	Open label, two-part (dose escalation, dose expansion), sequential assignment • datopotamab deruxtecan + *Imfinzi* +/- platinum chemotherapy US, Japan	• Primary endpoints: DLT, safety • Secondary endpoints: ORR, DOR, PFS, OS, PK, ADA	• FPCD: Q1 2021 • Data anticipated: 2022+

Datopotamab deruxtecan (TROP2 ADC)

NSCLC and other cancers

Trial	Population	Patients	Design	Endpoints	Status
Phase III **TROPION-Breast01**	Inoperable or metastatic HR+, HER2- breast cancer	700	Open-label, randomized • datopotamab deruxtecan • investigator's choice standard of care chemotherapy (eribulin, vinorelbine, capecitabine, gemcitabine)	• Primary endpoints: PFS, OS • Secondary endpoints: ORR, DoR, DCR, PL, ADA	• Initiating • Data anticipated: 2022+
Phase I **TROPION-PanTumor01** **NCT03401385** **Partnered**	Subjects with advanced solid tumours NSCLC TNBC HR+ BC HER2-negative gastric/GEJ oesophageal Urothelial SCLC	770	Open label, two-part (dose escalation, dose expansion), sequential assignment • datopotamab deruxtecan Japan, US	• Primary endpoints: DLT, safety • Secondary endpoints: PK, anti-tumour activity, ADA	• FPCD: Q1 2018 • Data anticipated: 2022+ • Early data readout (NSCLC): Q1 2021 • Early data readout (TNBC): Q2 2021

Oncology – early-stage development

AZD0466 (Bcl2/xL inhibitor)

Cancer

Trial	Population	Patients	Design	Endpoints	Status
Phase I **NCT04214093**	Advanced haematologic malignancies	9	Monotherapy dose escalation, consisting of two arms: • Arm A: patients with low risk for tumour lysis syndrome (solid tumours, lymphomas, myelomas) • Arm B: patients with high risk for tumour lysis syndrome (relapsed/refractory haem malignancies)	• Primary endpoint: safety • Secondary endpoints: PK, anti-tumour activity	• FPCD: Q4 2019 • Data anticipated: Q4 2021
Phase I/II **NCT04865419**	Advanced haematologic malignancies	64	Module 1 Part A: Dose escalation • AZD0466 Part B: Dose expansion • AZD0466 Module 2 - DDI trial AZD0466 with voriconazole	• Primary endpoint: safety • Secondary endpoints: PK, anti-tumour activity	• FPCD: Q2 2021 • Data anticipated: 2022+

MEDI1191 (IL12 modRNA)

Cancer

Trial	Population	Patients	Design	Endpoints	Status
Phase I **NCT03946800**	Advanced solid tumours	87	Open-label, dose-escalation and expansion trial of MEDI1191 administered intratumourally as monotherapy and in combination with *Imfinzi*	• Primary endpoints: safety and tolerability • Secondary endpoints: PK, immunogenicity and efficacy	• FPCD: Q2 2019 • Data anticipated: H2 2022

AZD1390 (ATM inhibitor)

Cancer

Trial	Population	Subjects	Design	Endpoints	Status
Phase I **NCT03423628**	Recurrent glioblastoma eligible for re-irradiation, brain metastases and leptomeningeal disease, newly-diagnosed glioblastoma patients	Up to 162	Open label trial • Arm A: recurrent GBM, AZD1390 +RT in dose escalation cohorts • Arm C: primary GBM, AZD1390 +RT in dose escalation cohorts	• Primary endpoints: safety, tolerability, MTD • Secondary endpoints: PK and preliminary assessment of anti-tumour activity	• FPCD: Q2 2018 • Data anticipated: 2022+

Adavosertib (WEE-1 inhibitor)

Ovarian cancer, uterine serous cancer, solid tumours

Trial	Population	Patients	Design	Endpoints	Status
Phase II **D6010C00004** **NCT02272790**	Platinum-resistant (PR) ovarian cancer	95	• Arm B: paclitaxel + adavosertib • Arm C: carboplatin + adavosertib Global trial	• Primary endpoint: ORR • Secondary endpoints: DoR, PFS, OS, DCR, safety and tolerability	• FPCD: Q1 2015 • LPCD: Q2 2018 • Data readout: Q3 2019
Phase II **D601HC00002** **NCT04590248**	Uterine serous carcinoma	120	• adavosertib monotherapy • Phase IIb, open-label, single-arm, multicentre study Global trial	• Primary endpoint: ORR • Secondary endpoints: DoR, depth of response, PFS	• FPCD: Q4 2020
Phase I **D6015C00002** **NCT02617277**	Advanced solid tumours	56	Dose escalation trial • adavosertib + *Imfinzi* US	• Primary endpoints: safety and tolerability, MTD	• FPCD: Q4 2015 • LPCD: Q4 2018 • Data readout: Q4 2019

AZD2811 (nanoparticle, Aurora B kinase inhibitor)

Cancer

Trial	Population	Patients	Design	Endpoints	Status
Phase II **NCT04745689**	Extensive-stage small cell lung cancer	100	Experimental: • AZD2811 + *Imfinzi* Induction: • *Imfinzi* + platinum chemotherapy (carboplatin or cisplatin & etoposide) Maintenance: • AZD2811 + *Imfinzi*	• Primary endpoint: PFS	• FPCD: Q1 2021 • Data anticipated 2022+
Phase I **NCT02579226**	Solid tumours	72	• Arm 1: AZD2811 (NP) dose escalation • Arm 2: AZD2811 (NP) dose expansion SCLC	• Primary endpoints: safety and tolerability, PK and efficacy	• FPCD: Q4 2015 • Data readout: Q2 2021

AZD4573 (CDK9 inhibitor)

Blood cancers

Trial	Population	Patients	Design	Endpoints	Status
Phase I/II **NCT04630756**	R/R haematologic malignancies	78	Open label, non-randomised trial Module 1 Part A: Dose setting • AZD4573 + *Calquence* (100mg twice daily) combination in DLBCL, all comers; ramp-up across 3 dose levels Module 1 Part B: Dose expansion • AZD4573 + *Calquence* (100mg twice daily) combination in GCB and non-GCB DLBCL i.v. route of administration 10 countries across North America, EU, ROW	• Primary endpoint Part A: safety • Primary endpoint Part B: ORR • Secondary endpoints Part A: safety, anti-tumour activity, PK	• FPCD: Q1 2021 • Data anticipated: 2022+
Phase I **NCT03263637**	R/R haematologic malignancies	45	• Arm 1: dose escalation in haematological malignancies excluding AML/ALL/high-risk MDS/CMML/CLL • Arm 2: dose escalation in relapsed or refractory AML, ALL, high-risk MDS, CMML, CLL and Richter's syndrome i.v. route of administration The Netherlands, UK, Germany	• Primary endpoints: safety, PK • Secondary endpoint: efficacy	• FPCD: Q4 2017 • Data anticipated: Q4 2021

AZD5305 (PARP1 inhibitor)

Solid tumours

Trial	Population	Patients	Design	Endpoints	Status
Phase I/IIa **NCT04644068**	Advanced, metastatic HER2-breast cancer (BRCAm, PALB2m or RAD51C/Dm) Advanced, metastatic TNBC PSR ovarian cancer (BRCAm, PALB2m or RAD51C/Dm) PSR ovarian cancer (HRD+) Prostate (mCRPC, BRCAm) Prostate (mCRPC, HRRm)	612	A modular, open-label, multicentre trial dose escalation • Module 1: AZD5305 • Module 2: AZD5305 + paclitaxel • Module 3: AZD5305 + carboplatin +/- paclitaxel	• Primary endpoints: safety and tolerability, PK • Secondary endpoints: efficacy	• FPCD: Q4 2020 • Data anticipated: 2022+

MEDI5752 (PD-1/CTLA-4 bispecific mAb)

Cancer

Trial	Population	Patients	Design	Endpoints	Status
Phase I/IIa **NCT03530397**	Advanced solid tumours	271	Open-label, dose-escalation and dose-expansion trial Dose escalation: MEDI5752 i.v. Dose expansion: MEDI5752 i.v. as monotherapy and in combination with chemotherapy • Arm A: MEDI5752 i.v. • Arm B: MEDI5752 i.v., pemetrexed and carboplatin • Arm C: pembrolizumab, pemetrexed and carboplatin	• Dose escalation primary endpoints: safety, MTD • Dose expansion primary endpoint: antitumour activity based on OR • Secondary endpoints: PK, ADA, tumoural baseline PD-L1, antitumour activity (OR, DoR, DCR, PFS, OS)	• FPCD: Q2 2018 • Data anticipated: 2022+
Phase Ib **NCT04522323**	Advanced renal cell carcinoma	70	Open-label, dose-escalation and dose-expansion trial • Arm 1: MEDI5752 and axitinib • Arm 2: MEDI5752 and lenvatanib	• Dose escalation primary endpoints: safety, MTD, RP2D & tolerability. Assess antitumour activity of the combination (ORR) • Secondary endpoints: PK, ADA and antitumour activity (PFS, OR, DoR, DCR, TTR, OS)	• FPCD: Q3 2020 • Data anticipated: 2022+

AZD5991 (MCL1 inhibitor)

Blood cancers

Trial	Population	Patients	Design	Endpoints	Status
Phase I/Ib/IIa **NCT03218683**	Relapsed/refractory haematologic malignancies	144	• Arm 1: AZD5991 dose escalation and expansion in R/R haematological malignancies • Arm 2: AZD5991 + venetoclax combination dose escalation in R/R AML i.v. route of administration US only	• Primary endpoint: safety • Secondary endpoints: PK, efficacy	• FPCD: Q3 2017 • Data anticipated: 2022+

Ceralasertib (AZD6738, ATR inhibitor)

Cancer

Trial	Population	Patients	Design	Endpoints	Status
Phase II **PLANETTE** **NCT04564027**	Solid tumours mCRPC	52	• Cohort A: ceralasertib; ATM-altered AST • Cohort B: ceralasertib; ATM-altered mCRPC	• Cohort A primary endpoint: ORR • Cohort B primary endpoint: Composite RR	• FPCD: Q1 2021 • Data anticipated: 2022+
Phase I **NCT02264678**	Solid tumours	250	• Arm 1: ceralasertib + carboplatin • Arm 2: ceralasertib dose escalation, ceralasertib + *Lynparza* • Arm 3: ceralasertib + *Imfinzi* North America, Europe and South Korea	• Primary endpoints: safety and tolerability, • PK and efficacy	• FPCD: Q4 2014 • Data anticipated: 2022+
Phase I **NCT03022409**	HNSCC	44	Window of opportunity • Arm 1: ceralasertib • Arm 2: *Lynparza* US, France, Taiwan and the UK	• Primary endpoint: Biomarker change	• FPCD: Q4 2017 • Data anticipated: Q4 2021

AZD7648 (selective DNA-PK inhibitor)

Advanced solid tumours

Trial	Population	Patients	Design	Endpoints	Status
Phase I **NCT03907969**	Advanced malignancies	234	Modular dose escalation and dose expansion trial • Arm 1: AZD7648 monotherapy • Arm 2: AZD7648 + pegylated liposomal doxorubicin • Arm 3: AZD7648 + *Lynparza* US, UK	• Primary outcomes: safety and tolerability • Secondary outcomes: PK, Cytochrome P450, preliminary anti-tumour activity	• FPCD: Q4 2019 • Data anticipated: 2022+

AZD8701 (FOXP3 antisense oligonucleotide)

Solid tumours

Trial	Population	Patients	Design	Endpoints	Status
Phase I/Ib **NCT04504669**	Advanced solid tumours	123	Dose escalation and dose expansion trial • Arm 1: AZD8701 monotherapy • Arm 2: AZD8701 & *Imfinzi* combination therapy i.v. route of administration US, CA, FR, ES	• Primary endpoints: safety & tolerability • Secondary endpoints: PK, PD, preliminary anti-tumour activity	• FPCD: Q3 2020 • Data anticipated: 2022+

MEDI9253 (rNDV-IL12)

Solid tumours

Trial	Population	Patients	Design	Endpoints	Status
Phase I **NCT04613492**	Advanced solid tumours	86	Open-label, dose-escalation and expansion trial • MEDI9253 + *Imfinzi*	• Primary endpoint: safety and tolerability • Secondary endpoints: PK, PD, immunogenicity and efficacy	• FPCD: Q4 2020 • Data anticipated: 2022+

IPH5201 (CD39 mAb)

Solid tumours

Trial	Population	Patients	Design	Endpoints	Status
Phase I **NCT04261075** **Partnered**	Advanced solid tumours	204	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. 4 countries - US and 3 in EU.	• Primary endpoints: AE, SAE, DLT • Secondary endpoints: OR, DC, PK, ADA	• FPCD: Q1 2020 • Data anticipated: 2022+

Oleclumab (CD73 mAb)

Cancer

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. US, Norway, Spain and Australian trial centres	• Primary endpoints: safety and anti-tumour activity • Secondary endpoints include: PFS, PK, immunogenicity, safety and anti-tumour activity	• FPCD: Q2 2018 • Data anticipated: Q4 2021

AZD2936 (PD-1/TIGIT Bispecific mAb)

Solid tumours

Trial	Population	Patients	Design	Endpoints	Status
Phase I **NCT04995523** **Partnered**	Non-Small-Cell Lung Carcinoma	147	Open-label, non-randomised dose-escalation and dose-expansion trial: • Part A: Dose escalation in checkpoint inhibitor (CPI) experienced NSCLC pts with AZD2936 Intravenous monotherapy • Part B: Dose expansion in checkpoint inhibitor (CPI) experienced NSCLC pts with AZD2936 intravenous monotherapy • Part C: Dose expansion in CPI Naive NSCLC pts with AZD2936 i.v. monotherapy • Part D: Dose expansion. Design to be confirmed by protocol amendment Europe, Australia, South Korea and North America	• Part A Dose escalation primary endpoints: safety, RP2D, MTD • Part B dose expansion primary endpoints: Safety and efficacy (ORR) • Part C dose expansion primary endpoints: Safety and efficacy (ORR) • Secondary endpoints: PK, PD (Receptor occupancy), efficacy (DCR, DoR, DRR, PFS)	• FPCD: Q4 2021 • Data anticipated: 2022+

AZD7789 (PD-1/TIM3 Bispecific mAb)

Cancer

Trial	Population	Patients	Design	Endpoints	Status
Phase I/IIa **NCT04931654**	Non-small-cell lung carcinoma Other tumours	81	Open-label, non-randomised dose-escalation and dose-expansion trial: • Part A: Dose escalation in post IO NSCLC pts with AZD7789 intravenous (iv) monotherapy • Part B: Dose expansion in post IO and IO naïve NSCLC pts with AZD7789 iv monotherapy. North America, Europe	• Primary endpoints: AE, SAE, DLTs, ORR • Secondary endpoints: ORR, DCR, DoR, PFS, OS, PK, ADA	• FPCD: Q4 2021 • Data anticipated: 2022+

BioPharmaceuticals – approved medicines and late-stage pipeline

Farxiga (SGLT2 inhibitor)

Heart failure and chronic kidney disease

Trial	Population	Patients	Design	Endpoints	Status
Phase III **DELIVER** **NCT03619213**	CHF patients with HFpEF	6100	• Arm 1: *Farxiga* 10mg QD • Arm 2: placebo Global trial - 21 countries	• Primary endpoint: time to the first occurrence of any of the components of the composite: CV death or hospitalisation for HF or an urgent HF visit	• FPCD: Q4 2018 • Data anticipated: H1 2022
Phase III **DAPA-MI** **NCT04564742**	Patients with 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 anticipated: 2022+
Phase I **NCT04856007**	Healthy Chinese volunteers	80	• Arm 1: *Farxiga* 5 mg + metformin 500 mg XR • Arm 2: *Farxiga*/metformin XR FDC 5/500 mg • Arm 3: *Farxiga* 10 mg + metformin 1000 mg XR • Arm 4: *Farxiga*/metformin XR FDC 10/1000 mg China only	• Primary endpoint: Plasma AUCinf, AUClast and Cmax of *Farxiga* and metformin respectively.	• FPCD: Q2 2021 • LPCD: Q2 2021 • Data anticipated: Q4 2021

Brilinta (P2Y12 receptor antagonist)

Cardiovascular risk reduction

Trial	Population	Patients	Design	Endpoints (primary)	Status
Phase III **THALES** **NCT03354429**	Patients with acute ischaemic stroke or transient ischaemic attack	11000	• Arm 1: *Brilinta* 90mg BID • Arm 2: placebo BID On a background of acetylsalicylic acid if not contra-indicated or not tolerated Global trial – 28 countries	• Primary endpoint: prevention of the composite of subsequent stroke and death at 30 days • Secondary endpoints include: prevention of subsequent ischaemic stroke at 30 days; reduction of overall disability at 30 days	• FPCD: Q1 2018 • LPCD: Q4 2019 • Data readout: Q1 2020 • Primary endpoint met

Lokelma (sodium zirconium cyclosilicate)

Hyperkalaemia

Trial	Population	Patients	Design	Endpoints	Status
Phase IIIb **DIALIZE China** **NCT04217590**	Patients with ESRD with hyperkalaemia and on stable haemodialysis	134	• Arm 1: *Lokelma* 5g QD for 8 weeks on non-dialysis days. Option to uptitrate to 10 and 15g QD. • Arm 2: placebo QD for 8 weeks on non-dialysis days China	• Primary endpoint: proportion of patients who maintain a pre-dialysis serum K between 4.0-5.0 mmol/L on 3 out of 4 dialysis treatments following the long interdialytic interval	• FPCD: Q4 2020 • Data anticipated: H1 2022
Phase III **HARMONIZE Asia** **NCT03528681**	Hyperkalaemia	250	Open-label *Lokelma* 10g TID for 48 hours followed by: • Arm 1: *Lokelma* 5g QD for 28 days • Arm 2: *Lokelma* 10g QD for 28 days • Arm 3: placebo QD for 28 days China, India	• Primary endpoint: maintenance of normokalaemia	• FPCD: Q2 2021 • Data anticipated: H2 2022
Phase III **DIALIZE-Outcomes** **NCT04847232**	Patients with recurrent hyperkalaemia on chronic haemodialysis	2300	• Arm 1: *Lokelma* 5g-15g QD for 4 weeks on non-dialysis days, thereafter adjusted monthly • Arm 2: placebo QD Global trial – 22 countries	• Primary endpoint: Time to first occurrence of SCD, stroke, or hospitalisation/intervention/ED visit due to arrhythmias	• FPCD: Q3 2021 • Data anticipated: 2022+
Phase III **STABILIZE-CKD** **NCT05056727**	Patients with CKD and hyperkalaemia or at risk of hyperkalaemia	1360	Open-label *Lokelma* (10g TID or 5g QD) for up to 72h, followed by 3 months open-label treatment with *Lokelma* (5g QOD to 15g QD) and uptitration of lisonopril or valsartan. Thereafter, patients are randomized to a 24 month treatment: • Arm 1: *Lokelma* (5g QOD to 15g QD) and lisonopril or valsartan • Arm 2: Placebo and lisonopril or valsartan	Primary endpoint: Total slope (eGFR measurements starting at randomisation) and chronic slope (eGFR measurements starting at 12 weeks after randomisation)	• FPCD: Q4 2021 • Data anticipated: 2022+

Roxadustat (HIF-PH inhibitor)

Anaemia

Trial	Population	Patients	Design	Endpoints	Status
Phase III **ANDES** **NCT01750190** **Partnered**	Anaemia in CKD in patients not receiving dialysis	922	• Arm 1: roxadustat • Arm 2: placebo Global trial	• Primary endpoint: haemoglobin response	• FPCD: Q4 2012 • LPCD: Q3 2018 • Data readout: Q4 2018 • Primary endpoint met Sponsored by FibroGen
Phase III **ALPS** **NCT01887600** **Partnered**	Anaemia in CKD in patients not receiving dialysis	597	• Arm 1: roxadustat • Arm 2: placebo Global trial	• Primary endpoint: haemoglobin response	• FPCD: Q2 2013 • LPCD: Q4 2017 • Data readout: Q3 2018 • Primary endpoint met Sponsored by Astellas
Phase III **DOLOMITES** **NCT02021318** **Partnered**	Anaemia in CKD in patients not receiving dialysis	616	• Arm 1: roxadustat • Arm 2: darbepoetin alfa Global trial	• Primary endpoint: haemoglobin response	• FPCD: Q1 2014 • LPCD: Q4 2019 • Data readout: Q1 2020 • Primary endpoint met Sponsored by Astellas
Phase III **OLYMPUS** **NCT02174627**	Anaemia in CKD in patients not receiving dialysis	2781	• Arm 1: roxadustat • Arm 2: placebo Global trial	• Primary efficacy endpoint: haemoglobin response • Primary safety objective: contribute CV safety data to pooled safety analyses across the Phase III program	• FPCD: Q3 2014 • LPCD: Q4 2018 • Data readout: Q4 2018 • Primary endpoint met Sponsored by AstraZeneca
Phase III **ROCKIES** **NCT02174731**	Anaemia in CKD in patients receiving dialysis	2133	• Arm 1: roxadustat • Arm 2: epoetin alfa Global trial	• Primary efficacy endpoint: haemoglobin response • Primary safety objective: contribute CV safety data to pooled safety analyses across the Phase III programme	• FPCD: Q3 2014 • LPCD: Q3 2018 • Data readout: Q4 2018 • Primary endpoint met Sponsored by AstraZeneca

Roxadustat (HIF-PH inhibitor)

Anaemia

Trial	Population	Patients	Design	Endpoints	Status
Phase III **SIERRAS** **NCT02273726** **Partnered**	Anaemia in CKD in patients receiving dialysis	741	• Arm 1: roxadustat • Arm 2: epoetin alfa Global trial	• Primary endpoint: haemoglobin response	• FPCD: Q4 2014 • LPCD: Q3 2018 • Data readout: Q4 2018 • Primary endpoint met Sponsored by FibroGen
Phase III **PYRENEES** **NCT02278341** **Partnered**	Anaemia in CKD in patients receiving dialysis	838	• Arm 1: roxadustat • Arm 2: epoetin alfa or darbepoetin alfa Global trial	• Primary endpoint: haemoglobin response	• FPCD: Q4 2014 • LPCD: Q3 2018 • Data readout: Q3 2018 • Primary endpoint met Sponsored by Astellas
Phase III **HIMALAYAS** **NCT02052310** **Partnered**	Anaemia in newly initiated dialysis patients	1043	• Arm 1: roxadustat • Arm 2: epoetin alfa Global trial	• Primary endpoint: haemoglobin response	• FPCD: Q4 2013 • LPCD: Q3 2018 • Data readout: Q4 2018 • Primary endpoint met Sponsored by FibroGen
Phase III **NCT03263091** **Partnered**	Anaemia in lower risk MDS patients	184	Open label roxadustat lead-in • Arm 1: roxadustat • Arm 2: placebo US/global trial	• Primary endpoint: proportion of patients achieving transfusion independence	• FPCD: Q3 2017 • Data anticipated: 2022+ Sponsored by FibroGen
Phase II/III **NCT03303066** **Partnered**	Anaemia in lower risk MDS patients	175	Open label roxadustat lead-in • Arm 1: roxadustat • Arm 2: placebo China	• Primary endpoint: haemoglobin response	• FPCD: Q2 2018 • Data anticipated: 2022+ Sponsored by FibroGen
Phase II **NCT04076943** **Partnered**	Anaemia in patients receiving chemotherapy treatment for non-myeloid malignancies	92	Open label trial • roxadustat 3x week 16 weeks US	• Primary endpoint: maximum change in haemoglobin within 16 weeks from baseline without RBC transfusion	• FPCD: Q3 2019 • LPCD: Q3 2020 • Data readout: Q2 2021 • Primary endpoint met Sponsored by FibroGen

Eklira/ Tudorza (LAMA, DPI)

COPD

Trial	Population	Number of patients	Design	Endpoints	Status
Phase I **NCT03276052**	Healthy Chinese volunteers	20	Open-label trial in healthy Chinese male and female participants. • aclidinium bromide 400 µg DPI - single and multiple twice daily doses China	• Primary endpoints: safety and tolerability, PK	• Initiating • Data anticipated: H1 2022

Duaklir Genuair (LAMA/LABA, DPI)

COPD

Trial	Population	Patients	Design	Endpoints	Status
Phase III **AVANT** **NCT03022097**	Patients with stable COPD	1060	• Arm 1: *Duaklir Genuair* 400/12 µg DPI • Arm 2: aclidinium bromide 400 µg DPI • Arm 3: formoterol fumarate 12 µg DPI • Arm 4: tiotropium 18 µg DPI Global trial – five countries	• Primary endpoints: • Change from baseline in one hour morning post-dose dose FEV1 *Duaklir Genuair* 400/12 µg compared to Aclidinium bromide at Week 24 • Change from baseline in morning pre-dose (trough) FEV1 of *Duaklir Genuair* 400/12 µg compared to Formoterol fumarate at Week 24 • Change from baseline in trough FEV1 of Aclidinium bromide 400 µg compared to placebo at Week 24	• FPCD: Q1 2017 • Data anticipated: H2 2022

Breztri, Trixeo (PT010, LAMA/LABA/ICS, pMDI)

Asthma

Trial	Population	Patients	Design	Endpoints	Status
Phase III **KALOS** **NCT04609878**	Severe asthma	2800	Randomised, double-blind, double dummy, parallel group and multicentre Treatments (24 to 52 week variable length) • BGF MDI 320/28.8/9.6µg BID pMDI • BGF MDI 320/14.4/9.6µg BID pMDI • BFF MDI 320/9.6µg BID pMDI • *Symbicort* 320/9µg BID pMDI Multi-country	• 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 24 • Primary endpoint of pooled trials D5982C00007 and D5982C00008: Rate of severe asthma exacerbations • Secondary endpoint: Change from baseline in morning pre-dose trough FEV1 at Week 24	• FPCD: Q1 2021 • Data anticipated: 2022+
Phase III **LOGOS** **NCT04609904**	Severe asthma	2800	Randomised, double-blind, double dummy, parallel group and multicentre Treatments (24 to 52 week variable length) • BGF MDI 320/28.8/9.6µg BID pMDI • BGF MDI 320/14.4/9.6µg BID pMDI • BFF MDI 320/9.6µg BID pMDI • *Symbicort* 320/9µg BID pMDI Multi-country	• 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 24 • Primary endpoint of pooled trials D5982C00007 and D5982C00008: Rate of severe asthma exacerbations • Secondary endpoint: Change from baseline in morning pre-dose trough FEV1 at Week 24	• FPCD: Q1 2021 • Data anticipated: 2022+

Daliresp/Daxas (PDE4 inhibitor, oral)

COPD

Trial	Population	Patients	Design	Endpoints	Status
Post Launch PASS NCT03381573	COPD	124080	A retrospective cohort trial comparing COPD patients aged 40 years and older with new exposure to roflumilast with up to 5 unexposed (i.e., not roflumilast-exposed) COPD controls matched by propensity score (PS), age, sex, and year of cohort entry. The trial is using electronic healthcare databases in the US (Military Health System database), Germany (German Pharmacoepidemiological Research Database), Sweden (national databases including healthcare, death, and demographics data) and Norway.	• Primary endpoint: all-cause mortality (up to five years)	• Data anticipated: 2022+

Fasenra (IL5R mAb)

Severe, uncontrolled asthma

Trial	Population	Patients	Design	Endpoints	Status
Phase III **MELTEMI** **NCT02808819**	A multicentre, open-label, safety extension trial with *Fasenra* for asthmatic adults on ICS plus LABA2 Agonist Age 18-75 years	447	• Arm 1: *Fasenra* 30mg Q4W s.c. • Arm 2: *Fasenra* 30mg Q8W s.c. Global trial - 15 countries	• Primary endpoint: safety and tolerability	• FPCD: Q2 2016 • LPCD: Q3 2019 • Data readout: Q3 2020 • Primary endpoint met
Phase IIIb **PONENTE** **NCT03557307**	Severe eosinophilic asthmatics receiving HD ICS + LABA and chronic OCS with or without additional asthma controller(s). Age 18 Years and older	598	• Arm 1: *Fasenra* 30mg Q8W s.c. 38-week trial Global trial – 16 countries	• Primary endpoint: reduction of oral corticosteroid dose	• FPCD: Q3 2018 • LPCD: Q3 2019 • Data readout: Q4 2020 • Primary endpoint met
Phase III **D3250C00036 China ICS/LABA Trial (MIRACLE)** **NCT03186209**	Severe, uncontrolled asthma, despite background controller medication, MD & HD ICS + LABA ± chronic OCS Age 12-75 years	666	• Arm 1: *Fasenra* 30mg Q8W s.c. • Arm 2: placebo s.c. 56-week trial Global trial – 4 countries	• Primary endpoint: annual asthma exacerbation rate • Secondary endpoints: assess pulmonary function, asthma symptoms, other asthma control metrics	• FPCD: Q4 2017 • Data readout: 2022+

Fasenra (IL5R mAb)

Severe, uncontrolled asthma

Trial	Population	Patients	Design	Endpoints	Status
Phase III **BORA** **NCT02258542**	Severe asthma, inadequately controlled despite background controller medication, MD & HD ICS/LABA ± chronic OCS Age 12-75 years	2133	Randomised double-blind safety extension trial • Arm 1: *Fasenra* 30mg Q4W s.c. • Arm 2: *Fasenra* 30mg Q8W s.c. 56-week (adults) 108-week (adolescents) Global trial – 24 countries	• Primary endpoint: safety and tolerability	• FPCD: Q4 2014 • Data readout: Q3 2018 • Primary endpoint met
Phase III **GREGALE** **NCT02417961**	Severe asthma, inadequately controlled despite background controller medication, MD & HD ICS + LABA ± chronic OCS Age 18-75 years	162	• Arm 1: *Fasenra* 30mg Q4W s.c. 28-week (adults) Global trial – two countries	• Primary endpoint: functionality, reliability, and performance of a pre-filled syringe with *Fasenra* administered at home	• FPCD: Q2 2015 • Data readout: Q2 2016 • Primary endpoint met
Phase III **ARIA** **NCT02821416**	Allergen-induced inflammation in mild, atopic asthma Age 18-65 years	46	A double-blind, randomised, parallel group, placebo-controlled multicentre trial to evaluate the effect of *Fasenra* on allergen-induced inflammation in mild, atopic asthma patients • Arm 1 : *Fasenra* 30mg Q4W s.c. • Arm 2: placebo Q4W s.c. 37-week trial	• Primary endpoint: safety and tolerability • Primary endpoint: the effect of *Fasenra* on allergen induced eosinophil changes in sputum and allergen-induced late asthmatic response	• FPCD Q4 2016 • LPCD: Q2 2019 • Data readout: Q4 2020 • Primary endpoint met
Phase III **ALIZE** **NCT02814643**	Adolescent and young adult patients with severe asthma receiving a seasonal influenza vaccine Ages 12-21 years	103	A multicentre, randomised, double-blind, parallel group, placebo-controlled trial • Arm 1: *Fasenra* 30mg Q4W s.c. with one dose of seasonal influenza virus vaccine i.m. • Arm 2: placebo Q4W s.c. with one dose of seasonal influenza virus vaccine i.m. 12-week trial	• Primary endpoints: • Post-dose strain-specific HAI) antibody GMFRs • Post-dose strain-specific serum HAI antibody GMTs • Proportion of patients who experience a strain-specific post-dose antibody response with antibody response defined as a ≥4-fold rise in HAI antibody titre	• FPCD: Q3 2016 • Data readout: Q3 2017 • Primary endpoint met

Fasenra (IL5R mAb)

Severe, uncontrolled asthma, COPD and other eosinophilic diseases

Trial	Population	Patients	Design	Endpoints	Status
Phase III **GRECO** **NCT02918071**	Severe asthma on ICS/LABA Age 18-75 years	121	Open label • *Fasenra* 30mg Q4W 28-week trial Global trial - two countries	• Primary endpoint: percentage of patients/caregivers who successfully self administer at home	• FPCD: Q4 2016 • Data readout: Q4 2017 • Primary endpoint met
Phase IIIb **ANDHI** **NCT03170271**	Patients with severe asthma uncontrolled on SoC treatment. Age 18-75	659	• Arm 1: *Fasenra* 30mg Q8W s.c. • Arm 2: placebo Q8W s.c. 24-week trial Global trial – 15 countries	• Primary endpoint: rate of asthma exacerbations • Secondary outcome measures: Saint George Respiratory Questionnaire (SGRQ)	• FPCD: Q3 2017 • LPCD: Q1 2019 • Data readout: Q4 2019 • 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 40-85 years	868	Double-blind, placebo controlled • Arm 1: *Fasenra* 100mg Q8W s.c. • Arm 2: placebo Q8W s.c. 56-week treatment Global trial – 26 countries	• Primary endpoint: annualized rate of moderate or severe exacerbations over 56 weeks	• FPCD Q4 2019 • Data anticipated: 2022+
Phase III **MAHALE** **NCT05006573**	Patients With non-cystic fibrosis bronchiectasis (NCFB) with eosinophilic inflammation Age 18 years and older	420	Double blind treatment period and open label extension trial • Arm 1: *Fasenra* 30mg Q4W s.c. • Arm 2: placebo Q4W s.c. 52-week Global trial – 17 countries	• Primary endpoint: annualised bronchiectasis exacerbation rate at week 52	• FPCD: Q3 2021 • Data anticipated: 2022+
Phase I **AMES** **NCT02968914**	Healthy volunteers age 18-55 years	180	Open label trial • *Fasenra* 30 mg PK administered by APFS device • *Fasenra* 30 mg PK administered by AI device 8-week trial Global trial – two countries	• Primary endpoint: PK comparability	• FPCD: Q1 2017 • Data readout: Q3 2017

Fasenra (IL5R 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 standard of care therapy Age 18-75 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 18-75 years	148	• Arm 1: *Fasenra* 30mg Q8W s.c. • Arm 2: placebo Q8W s.c. 56-week trial Global trial - 10 countries	• Primary endpoint: Change in endoscopic total nasal polyp score and change in mean nasal blockage score	• FPCD: Q4 2019 • Data anticipated: 2022+
Phase III **MANDARA** **NCT04157348**	Patients with relapsing or refractory 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 both weeks 36 and 48.	• FPCD: Q4 2019 • Data anticipated: 2022+
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- 9-12 countries	• Primary endpoint: Time to first HES worsening/flare	• FPCD: Q3 2020 • Data anticipated: H2 2022

Fasenra (IL5R mAb)

Gastrointestinal diseases

Trial	Population	Patients	Design	Endpoints	Status
Phase III **MESSINA** **NCT04543409**	Documented diagnosis of EoE Age 12 to 65 years	170	Double blind treatment period and open label period(s) • Arm 1: *Fasenra* 30mg Q4W s.c. • Arm 2: placebo Q4W s.c. 24-week Global trial – 12 countries	• Primary endpoints: histologic response at week 24, change from baseline in DSQ score at week 24	• FPCD: Q4 2020 • Data anticipated: H2 2022
Phase III **HUDSON**	Patients with eosinophilic gastritis and/or gastroenteritis. Age >=12yrs	220	Double blind treatment period and open label extension • Arm 1: *Fasenra* s.c. • Arm 2: placebo s.c. 24-week Global trial	• Dual primary endpoints at week 24: • Proportion of patients achieving a histological response in the stomach and/or in the duodenum • Absolute change in symptoms of EG/EGE	• Initiating • Data anticipated: 2022+

Fasenra (IL5R mAb)

Dermatology

Trial	Population	Patients	Design	Endpoints	Status
Phase III **FJORD** **NCT04612790**	Patients with symptomatic (newly diagnosed or relapsing) bullous pemphigoid	120	Double blind treatment period and open label period • Arm 1: *Fasenra* • Arm 2: placebo 36-week Global trial	• Primary endpoint: Proportion of patients with complete sustained (≥2 months) remission off OCS at 36 weeks	• FPCD: Q2 2021 • Data anticipated: 2022+
Phase II **ARROYO** **NCT04612725**	Patients with moderate/severe chronic spontaneous urticaria, and resistant to H1 treatment	160	Double blind treatment period and open label period • Arm 1: *Fasenra* regimen 1 • Arm 2: *Fasenra* regimen 2 • Arm 3: placebo 24-week Global trial	• Primary endpoint: Change from baseline in ISS7 at week 12	• FPCD: Q4 2020 • Data anticipated: H2 2022
Phase II **HILLIER** **NCT04605094**	Patients with moderate to severe atopic dermatitis despite treatment with topical medications	160-200	Double blind treatment period and open label periods • Arm 1: *Fasenra* • Arm 2: placebo 16-week Global trial	• Primary endpoint: Proportion of patients with an IGA 0/1 and a decrease in IGA of ≥ 2 points at week 16	• FPCD: Q4 2020 • Data anticipated: H2 2022

Tezepelumab (TSLP mAb)

Severe, uncontrolled asthma

Trial	Population	Patients	Design	Endpoints	Status
Phase III NAVIGATOR NCT03347279 Partnered	Severe asthma Age 12-80 years	1061	• Arm 1: tezepelumab 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), asthma control (ACQ-6)	• FPCD: Q1 2018 • LPCD: Q3 2019 • Data readout: Q4 2020 • Primary endpoint met
Phase III SOURCE NCT03406078 Partnered	Severe asthma Age 18-80 years	150	• Arm 1: tezepelumab s.c. • Arm 2: placebo s.c. 48 week trial Global trial – seven countries	• Primary endpoint: Reduction from baseline in daily OCS dose while not losing asthma control • Secondary endpoint: Annual asthma exacerbation rate	• FPCD: Q2 2018 • LPCD: Q4 2019 • Data readout: Q4 2020 • Primary endpoint not met
Phase III DESTINATION NCT03706079 Partnered	Severe asthma Age 12-80 years	951	Extension trial to NAVIGATOR and SOURCE • Arm 1: tezepelumab s.c. • Arm 2: placebo s.c. 52 week trial (subjects from NAVIGATOR); 56 week trial (subjects from SOURCE) Global trial – 18 countries	• Primary endpoint: Exposure adjusted rates of AEs/SAEs • Secondary endpoints: Annual asthma exacerbation rate	• FPCD: Q1 2019 • LPCD: Q4 2020 • Data anticipated: H2 2022
Phase III PATH-HOME NCT03968978 Partnered	Severe asthma Age 12-80 years	216	• Arm 1: tezepelumab s.c. via AI • Arm 2: tezepelumab s.c. via APFS 24 week trial Global trial – 4 countries	• Primary endpoint: Proportion of health care professionals and patients /caregivers who successfully administrated tezepelumab in clinic and at home with an APFS or an AI, respectively	• FPCD: Q2 2019 • LPCD: Q3 2019 • Data readout: Q4 2020 • Primary endpoint met

Tezepelumab (TSLP mAb)

Severe, uncontrolled asthma, COPD & CRSwNP

Trial	Population	Patients	Design	Endpoints	Status
Phase III **WAYPOINT** **NCT04851964** **Partnered**	Severe chronic rhinosinusitis with nasal polyps (CRSwNP) Age 18+	400	• Arm 1: tezepelumab s.c. • Arm 2: placebo s.c. 52 week trial Global trial – 11 countries	• Co-primary endpoint: nasal polyp score and participant reported nasal congestion	• FPCD: Q2 2021 • Data anticipated: 2022+
Phase III **DIRECTION** **NCT03927157** **Partnered**	Severe asthma Age 18-80 years	396	• Arm 1: tezepelumab s.c. • Arm 2: placebo s.c. 52 week trial Regional Asia trial – three countries	• Primary endpoint: annual asthma exacerbation rate • Secondary endpoints: change from baseline in pre-BD FEV1, asthma related QoL (AQLQ(S)+12), asthma control (ACQ-6)	• FPCD: Q3 2019 • Data anticipated: 2022+
Phase III **NOZOMI** **NCT04048343** **Partnered**	Severe asthma 12-80 years	65	• Arm 1: tezepelumab s.c. 52 week trial Local trial - Japan	• Primary endpoint: number of patients with adverse events	• FPCD: Q2 2019 • LPCD: Q4 2019 • Data readout: Q2 2021
Phase II **CASCADE** **NCT03688074** **Partnered**	Severe asthma Age 18-75 years	116	• Arm 1: tezepelumab s.c. • Arm 2: placebo s.c. 28 week trial Global trial – five countries	• Primary endpoint: number of airway submucosal inflammatory cells/mm2 of bronchoscopic biopsies	• FPCD: Q4 2018 • LPCD: Q4 2019 • Data readout: Q2 2021 • Primary endpoint met
Phase IIa **COURSE** **NCT04039113** **Partnered**	Moderate to very severe COPD Age 40-80	338	• Arm 1: tezepelumab 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 • Data anticipated: 2022+

PT027 (SABA/ICS, pMDI)

Asthma

Trial	Population	Patients	Design	Endpoints	Status
Phase III **MANDALA** **NCT03769090** **Managed by Avillion**	Moderate to severe asthma	3100	Randomised, double-blind, multicentre, parallel group Treatments (minimum 24-week treatment period) • BDA (budesonide albuterol) MDI 80/180 µg prn • BDA MDI 160/180 µg prn • AS (albuterol sulphate) MDI 180 µg prn Multi-country	• 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**	Mild to moderate asthma	1000	Randomised, double-blind, multicentre and parallel-group Treatments (12 week treatment period) • BDA MDI 80/180 µg QID • BDA MDI 160/180 µg QID • BD MDI 160 µg QID • AS MDI 180 µg QID • placebo MDI QID Multi-country	• 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 III **TYREE** **NCT04234464** **Managed by Avillion**	Asthma with exercise induced bronchoconstriction	60	Randomised, double-blind, multicentre crossover Treatments (single dose) • BDA MDI 160/180 µg • placebo MDI QID US	• Primary endpoint: The maximum percentage fall from post-dose, pre-exercise baseline in forced expiratory volume in 1 second (FEV1) observed up to 60 minutes post-exercise challenge	• FPCD: Q1 2020 • LPCD: Q3 2020 • Data readout: Q4 2020 • Primary endpoint met

Saphnelo (type I interferon receptor mAb)

Lupus (SLE / LN)

Trial	Population	Patients	Design	Endpoints	Status
Phase III **TULIP-1 SLE** **NCT02446912**	Moderate to severe SLE	450	• Arm 1: 300mg i.v. anifrolumab Q4W for 48 weeks • Arm 2: 150mg i.v. anifrolumab Q4W for 48 weeks • Arm 3: placebo i.v. Q4W for 48 weeks	• Primary endpoint: response in SLE responder index at week 52	• FPCD: Q4 2015 • LPCD: Q4 2017 • Data readout: Q3 2018 • Primary endpoint not met
Phase III **TULIP-2 SLE** **NCT02446899**	Moderate to severe SLE	360	• Arm 1: 300mg i.v. anifrolumab Q4W for 48 weeks • Arm 2: placebo i.v. Q4W for 48 weeks	• Primary endpoint: response in SLE responder index at week 52 BICLA at week 52	• FPCD: Q4 2015 • LPCD: Q4 2017 • Data readout: Q3 2019 • Primary endpoint met
Phase III **TULIP LTE** **NCT02794285**	Moderate to severe SLE	630	• Arm 1: 300mg i.v. anifrolumab Q4W for 152 weeks • Arm 2: placebo i.v. Q4W for 152 weeks	• Primary endpoint: extension to evaluate long-term safety and tolerability	• FPCD: Q2 2016 • LPCD: Q4 2018 • Data anticipated: H1 2022
Phase II **TULIP-LN1** **NCT02547922**	Active proliferative LN	150	• Arm 1: 900 mg i.v. Q4W for 12 weeks then 300mg i.v. anifrolumab Q4W for 36 weeks • Arm 2: 300 mg i.v. anifrolumab Q4W for 48 weeks • Arm 3: placebo i.v. Q4W for 48 weeks	• Response in proteinuria at week 52	• FPCD: Q4 2015 • LPCD: Q4 2018 • Primary endpoint not met

Saphnelo (type I interferon receptor mAb)

Lupus (SLE)

Trial	Population	Patients	Design	Endpoints	Status
Phase III **TULIP-SC** **NCT04877691**	Moderate to severe SLE patients	360	• Arm 1: *Saphnelo s.c.* • Arm 2: placebo Global	• Primary endpoint: reduction in overall disease activity, as measured by BICLA at week 52	• FPCD: Q3 2021 • Data anticipated: 2022+
Phase III **AZALEA-SLE** **NCT04931563**	Moderate to severe SLE patients	328	• Arm 1: 300 mg *Saphnelo* i.v. Q4W • Arm 2: 300 mg placebo i.v.Q4W Asia	• Primary endpoint: BICLA response at week 52	• FPCD: Q4 2021 • Data anticipated: 2022+

Brazikumab (IL23 inhibitor)

Inflammatory bowel disease (Crohn's disease, ulcerative colitis)

Trial	Population	Patients	Design	Endpoints	Status
Phase IIb / III **INTREPID** **NCT03759288**	Crohn's disease	928	Stage 1 • Arm 1: brazikumab high i.v. dose on day 1, 29 and 57 + s.c. brazikumab on day 85 and every 4 weeks through week 48 • Arm 2: brazikumab low i.v. dose on day 1, 29 and 57 s.c. brazikumab on day 85 and every 4 weeks through week 48 • Arm 3: placebo Stage 2 • Arm 1: brazikumab high i.v. dose on day 1, 29 and 57 + s.c. brazikumab on day 85 and every 4 weeks through week 48 • Arm 2: brazikumab low i.v. dose on day 1, 29 and 57 s.c. brazikumab on day 85 and every 4 weeks through week 48 • Arm 3: adalimumab s.c. on day 1, 15, 29 and every 2 weeks through week 50	• Stage 1 primary endpoint: percentage of patients with CDAI remission at week 12 • Stage 1 secondary endpoints: Percentage of patients with endoscopic response at week 12, Percentage of patients with clinical remission at week 12 • Stage 2 primary endpoints: Percentage of patients with endoscopic response at week 52; Percentage of patients with clinical remission at week 52 • Stage 2 secondary endpoints: percentage of patients with endoscopic response at both Week 12 and Week 52; percentage of patients with clinical remission at both Week 12 and Week 52	• FPCD: Q4 2018 • Data anticipated: 2022+
Phase III **NCT03961815**	Crohn's Disease	161	Open label extension	• Primary endpoint: safety of long-term treatment with brazikumab	• FPCD: Q2 2019 • Data anticipated: 2022+
Phase II **EXPEDITION** **NCT03616821**	Ulcerative colitis	256	• Arm 1: brazikumab dose 1 i.v. on day 1, 15 and 43 + s.c. brazikumab from day 71 and every 4 weeks • Arm 2: brazikumab dose 2 i.v. on day 1, 15 and 43 + s.c. brazikumab from day 71 and every 4 weeks • Arm 3: placebo	• Primary endpoint: clinical remission at week 10 • Secondary endpoint: sustained clinical remission at week 10 and 54	• FPCD: Q3 2018 • Data anticipated: 2022+
Phase II **NCT04277546**	Ulcerative colitis	165	Open label extension	• Clinically significant adverse events	• FPCD: Q1 2020 • Data anticipated: 2022+
Phase I **NCT05033431**	Healthy volunteers	48	Open-label Study to Evaluate the Pharmacokinetics, Safety and Tolerability of a Single Dose of Brazikumab Administered by IV Infusion and SC Injection in Healthy Chinese and White Participants	• Primary endpoints to be evaluated in healthy Chinese and White participants: • Cmax of brazikumab • AUCinf of brazikumab • AUClast of brazikumab • AUC0-28d of brazikumab	• FPCD: Q4 2021 • Data anticipated: H2 2022

Nirsevimab (Respiratory syncytial virus mAb-YTE)

Infection

Trial	Population	Patients	Design	Endpoints	Status
Phase III **MELODY** **NCT03979313**	Healthy infants (born 35 weeks 0 days or greater GA)	3000 (total) 1500 (efficacy cohort) 1500 (safety cohort)	Randomised, Double-blind, placebo-controlled Arm 1: nirsevimab i.m. Arm 2: placebo i.m. Global trial – 31 countries	• Primary endpoints: efficacy • Secondary endpoints: safety, PK, ADA	• FPCD: Q3 2019 (efficacy cohort) • LPCD: Q1 2020 (efficacy cohort) • Data readout: Q2 2021 (efficacy cohort) • Primary endpoint met • FPCD: Q2 2021 (safety cohort) • Data anticipated: H2 2022 (safety cohort)
Phase III **CHIMES** **NCT05110261**	Healthy infants (born 29 weeks 0 days or greater GA)	800 (TBC	Randomised, Double-blind, placebo-controlled Arm 1: nirsevimab i.m. Arm 2: placebo i.m. China Only	• Primary endpoints: efficacy • Secondary endpoints: safety, PK, ADA	• FPCD: Q4 2021 • LPCD: 2022+ • Data readout: 2022+
Phase II/III **MEDLEY** **NCT03959488**	High risk preterm (born 35 weeks 0 day or less GA), CHD and CLD infants eligible to receive *Synagis*	925	Randomised, Double-blind, palivizumab-controlled Arm 1: nirsevimab i.m. Arm 2: *Synagis* i.m. Global trial – 32 countries	• Primary endpoints: safety and tolerability • Secondary endpoints: PK, ADA and descriptive efficacy	• FPCD: Q3 2019 • LPCD: Q4 2020 • Data readout: Q2 2021 • Safety objective met
Phase IIb **NCT02878330**	29-35 WK GA (gestational age) infants	1453	Randomised, double-blind, placebo-controlled trial Arm 1: nirsevimab 50mg i.m. Arm 2: placebo i.m.	• Primary endpoints: safety and efficacy	• FPCD: Q4 2016 • LPCD: Q4 2017 • Data readout: Q4 2018 • Primary endpoint met
Phase II **Global IC** **NCT04484935**	Immunocompromised children who are ≤ 24 months of age at the time of dose administration	100	Open-label, Uncontrolled, single-dose trial • nirsevimab i.m. Route of administration: i.m.	• Primary endpoints: safety and tolerability • Secondary endpoints: PK, ADA, efficacy	• FPCD: Q3 2020 • Data anticipated: 2022+
Phase I **China** **NCT04840849**	Healthy Chinese adults, 18-45 years of age	24	Randomised, Double-blind, placebo-controlled Arm 1: nirsevimab 50mg i.m. Arm 2: placebo i.m. Route of administration: i.m. China only	• Primary endpoint: PK • Secondary endpoints: ADA, safety	• FPCD: Q2 2021 • LPCD: Q2 2021 • Data anticipated: H1 2022

AZD1222/AZD2816 (SARS-CoV-2)

Prevention of COVID-19

Trial	Population	Patients	Design	Endpoints	Status
Phase II/III **COV002 (UK)** **NCT04400838** **Partnered**	Main efficacy trial: healthy adults aged ≥18 years Healthy adults 56 - <70 years Healthy adults ≥70 years Healthy children 5 – 12 years	10812	Single-blinded, randomised, controlled, multicentre trial with sequential age escalation/de-escalation immunogenicity sub-studies that include prime boost • AZD1222 • Control vaccine: MenACWY UK	• Primary endpoint: efficacy and safety • Secondary endpoints: safety, tolerability, reactogenicity, and immunogenicity	• FPCD: Q2 2020 • LPCD: Q4 2020
Phase III **D8110C00001 (US, global)** **NCT04516746**	Healthy adults Aged 18-65 years	32429	Adaptive, double-blinded, randomised placebo-controlled trial • AZD1222 • placebo US, Peru, Chile	• Primary endpoints: efficacy, safety, tolerability, and reactogenicity • Secondary endpoints: immunogenicity	• FPCD: Q3 2020 • LPCD: Q1 2021 • Data readout: Q1 2021
Phase III **COV003 (Brazil)** **NCT04536051** **Partnered**	Health professionals and adults with high potential for exposure to SARS-CoV-2 Age 18-55 years	10416	Single-blinded, randomised, controlled multicentre trial • AZD1222 • Control vaccine: MenACWY Brazil	• Primary endpoint: efficacy • Secondary endpoints: safety, tolerability, reactogenicity, and immunogenicity	• FPCD: Q2 2020 • LPCD: Q4 2020
Phase II/III AZD2816 (UK, Brazil, South Africa, Poland) **NCT04973449**	Healthy adults Aged > 18 years	2590	Partially double-blinded, randomized, active-controlled trial in unvaccinated or previously vaccinated participants • AZD2816 • AZD1222	• Primary endpoint: safety, tolerability • Secondary endpoint: immunogenicity	• FPCD: Q2 2021
Phase I/II **COV001 (UK)** **NCT04324606** **Partnered**	Healthy adults Age 18-55 years	1077	Single-blinded, randomised, controlled, multicentre trial • AZD1222 • Control vaccine: MenACWY UK	• Primary endpoint: efficacy and safety • Secondary endpoints: safety, tolerability, reactogenicity, and immunogenicity	• FPCD: Q2 2020 • LPCD: Q2 2020
Phase I/II **COV005 (SA)** **NCT04444674** **Partnered**	Healthy adults Age 18-65 years HIV+ subgroup	2130	Adaptive, double-blinded, randomised placebo-controlled trial • AZD1222 • placebo South Africa	• Primary endpoint: efficacy, safety, and immunogenicity	• FPCD: Q2 2020 • LPCD: Q4 2020

AZD1222/AZD2816 (SARS-CoV-2)

Prevention of COVID-19

Trial	Population	Patients	Design	Endpoints	Status
Phase I/II **D8111C00002** **NCT04568031**	Healthy adults Age ≥18 years	256	Double-blinded, randomised, placebo-controlled multicentre trial • AZD1222 • placebo Japan	• Primary endpoints: safety, tolerability, reactogenicity, immunogenicity • Secondary endpoints: immunogenicity	• FPCD: Q3 2020 • LPCD: Q4 2020 • Data readout: Q4 2020
Phase I/II **COV004 (Kenya)** **Partnered**	Healthy adults	400	Double-blinded, randomised, placebo-controlled multicentre trial • AZD1222 • Control vaccine: rabies Kenya	• Primary endpoints: safety, tolerability, reactogenicity, immunogenicity • Secondary endpoints: immunogenicity	• FPCD: Q4 2020 • LPCD: Q3 2021
Phase II **COV006 (UK)** ISRCTN15638344 **Partnered**	Healthy children and adolescents age 6-17 years	261	Single-blinded, randomized multicentre trial • AZD1222 • Meningococcal Group B vaccine UK	• Primary endpoints: safety, tolerability, reactogenicity • Secondary endpoints: immunogenicity	• FPCD: Q1 2021 • LPCD: Q2 2021
Phase I **COV008 (UK)** **NCT04816019** **Partnered**	Healthy adults Age 18-55	54	Open label, randomized, dose-escalation trial of AZD1222 administered intranasally • AZD1222 UK	• Primary endpoint: safety, tolerability • Secondary endpoint: immunogenicity	• FPCD: Q1 2021 • LPCD: Q4 2020

AZD7442 (LAAB combination of AZD8895 & AZD1061)

Prevention and treatment of COVID-19

Trial	Population	Patients/Subjects	Design	Endpoints	Status
Phase III **PROVENT** **D8850C00002** **NCT04625725**	Adults having increased risk for inadequate response to active immunisation or having increased risk for SARS-CoV-2 infection	5197	Double-blinded, randomised, placebo controlled, multi centre study to determine safety and efficacy in pre-exposure prophylaxis • Arm 1: AZD7442 • Arm 2: placebo AZD7442/placebo (2:1) USA, UK, Belgium, France, Spain	• Primary endpoint: positive symptomatic illness post –dose • Secondary endpoints: Incidence of: nucleocapsid antibodies, emergency visits, PCR positive, ADA to AZD7442 in serum and AZD7442 serum conc.	• FPCD: Q4 2020 • LPCD: Q1 2021 • Data readout: Q3 2021 • Primary endpoint met
Phase III **STORM CHASER** **D8850C00003** **NCT04625972**	Adults with potential exposure to an identified individual with confirmed SARS-COV2 infection and at risk of developing COVID-19	1121	Double-blinded, randomised, placebo controlled, multi centre study to determine safety and efficacy in post-exposure prophylaxis • Arm 1: AZD7442 • Arm 2: placebo AZD7442/placebo (2:1) USA and UK	• Primary endpoint: positive symptomatic illness post –dose • Secondary endpoints: Incidence o: nucleocapsid antibodies, COVID-19 related death, all cause mortality, ADA to AZD7442 in serum and AZD7442 serum conc.	• FPCD: Q4 2020 • LPCD: Q1 2021 • Data readout: Q2 2021 • Primary endpoint not met
PHASE III **TACKLE** **NCT04723394**	Adults with confirmed mild to moderate SARS-COV2 infection. Symptomatic patients with documented positive SARS-Cov-2 molecular test	910	Double-blinded, randomised, placebo controlled, multi centre study to determine safety and efficacy for treatment of Covid-19 in non-hospitalised patients • Arm 1: AZD7442 • Arm 2: placebo AZD7442/placebo (1:1) UK, Germany, Spain, Italy, Hungary, Russia, US, Mexico, Japan, Poland, Czech Republic, Argentina, Brazil, and Ukraine	• Primary endpoint: efficacy in the prevention of the composite endpoint of either severe COVID-19 or death from any cause through study day 29 • Secondary endpoints: A composite of either death from any cause or hospitalisation for COVID-19 complications or sequelae (Day 1 to Day 169). Determine symptom severity and prevention of respiratory failure	• FPCD: Q1 2021 • LPCD: Q3 2021 • Data readout: Q4 2021 • Primary endpoint met
Phase I **NCT04507256**	Healthy adults Aged 18-55 years	60	Double-blinded, randomised, placebo controlled, single ascending dose trial • Arm 1: AZD7442 • Arm 2: placebo AZD7442/placebo (10:2) UK	• Primary endpoint: safety, tolerability and PK • Secondary endpoint: immunogenicity	• FPFD: Q3 2020 • LPCD: Q3 2020

Other COVID-19 trials

Treatment of COVID-19

Trial	Population	Patients/Subjects	Design	Endpoints	Status
Phase IIIa TACTIC-COVID NCT04355637	COVID-19	300	• Current SoC or SoC + *Pulmicort*	• Primary outcome measures: proportion of patients in both arms fulfilling the criteria for treatment failure	• FPCD: Q2 2020 • Data anticipated: Q2 2021
Phase IIIa STOIC NCT04416399	COVID-19	478	• Current SoC or SoC + *Pulmicort*	• Primary outcome measures: emergency department attendance of hospitalisation related to COVID-19	• FPCD: Q2 2020 • Data readout: Q1 2021
Phase IIIa INHASCO NCT04331054	COVID-19	436	• Current SoC or SoC + *Symbicort*	• Primary outcome measures: time (in days) to clinical improvement within 30 days after randomisation	• FPCD: Q2 2020 • Data anticipated: Q2 2021
Phase II TACTIC-E NCT04393246	COVID-19	1407	• Current SoC or current SoC + *Farxiga* + ambrisentan	• Primary outcome measures: time to incidence of the composite endpoint of: death, mechanical ventilation, extracorporeal membrane oxygenation, cardiovascular organ support, or renal failure	• FPCD: Q4 2020 • Data anticipated: H1 2022
Phase II ACCORD	COVID-19	180	• Current SoC or current SoC + tozorakimab (MEDI3506)	• Primary endpoints: time to a 2-point improvement on a 9-point category ordinal scale, discharge from hospital, or considered fit for discharge whichever comes first by Day 29	• FPCD: Q2 2020 • Data anticipated: Q4 2021

BioPharmaceuticals – early-stage development

Cotadutide (GLP-1-glucagon agonist)

Diabetes/CKD, NASH

Trial	Population	Patients	Design	Endpoints	Status
Phase II **NCT03555994**	Adults with type-2 diabetes	44	• Part A: cotadutide or placebo s.c. • Part B: cotadutide s.c. or placebo s.c. or liraglutide s.c. Sweden, Netherlands, UK	• Primary endpoint: change in hepatic glycogen concentration postprandially, adjusted by liver volume • Secondary endpoints: safety, tolerability, immunogenicity	• FPCD: Q2 2018 • Part A LPCD: Q4 2018 • Data readout: Q1 2019 • Part B FPCD: Q1 2020 • LPCD: Q1 2021 • Data readout: Q2 2021
Phase IIb **NCT03596177**	Overweight and obese patients with type-2 diabetes	27	• Cotadutide or placebo s.c. UK	• Primary endpoints: efficacy body weight loss • Secondary endpoint: change in total energy intake; change in total energy expenditure, active energy expenditure, resting energy expenditure; safety	• FPCD: Q4 2018 • LPCD: Q4 2019 • Data readout: Q4 2020
Phase IIb **NCT04019561**	Obese patients with non-alcoholic fatty liver disease (NAFLD)/non-alcoholic steatohepatitis (NASH)	74	• Arm 1: cotadutide high dose s.c. • Arm 2: placebo high dose s.c. • Arm 3: cotadutide low dose s.c. • Arm 4: placebo low dose s.c. US	• Primary endpoints: safety and tolerability • Secondary endpoints: change in hepatic fat fraction; change in liver fat volume; change in visceral adipose tissue	• FPCD: Q4 2019 • Data anticipated: Q4 2021
Phase II **NCT04515849**	Chronic kidney disease with type 2 diabetes mellitus	225	• Arm 1: cotadutide 100 micrograms • Arm 2: cotadutide 300 micrograms • Arm 3: cotadutide 600 micrograms • Arm 4: semaglutide • Arm 5: placebo	• Primary endpoint: efficacy change in UACR • Secondary endpoints: Change in HbA1c; change in glucose measured by CGM; effects on body weight; safety, tolerability, Immunogenicity	• FPCD: Q4 2020 • LPCD: Q3 2021 • Data anticipated: H2 2022
Phase I **NCT04091373**	Healthy adult patients	36		• Primary endpoint: exposure following a single s.c. of cotadutide at each of 3 different sites of injection • Secondary endpoints: immunogenicity; safety and tolerability	• FPCD: Q4 2019 • LPCD: Q1 2020 • Data readout: Q4 2020

AZD2373

Chronic kidney disease

Trial	Population	Patients	Design	Endpoints	Status
Phase I **NCT04269031**	Healthy volunteers	48	SAD. Dose escalation in 6 cohorts with 6 volunteers receiving AZD2373 and 2 volunteers receiving placebo in each cohort • Arm 1: AZD2373 • Arm 2: placebo US	• Primary endpoints: safety and tolerability • Secondary endpoint: PK parameters	• FPCD: Q1 2020 • LPCD: Q3 3021 • Data anticipated: H1 2022

AZD2693 (antisense oligonucleotide)

NASH

Trial	Population	Patients	Design	Endpoints	Status
Phase I **NCT04142424**	Healthy volunteers	62	SAD. 6 cohorts with 6 volunteers receiving AZD2693 and 2 volunteers receiving placebo in each cohort • Arm 1: AZD2693 s.c. • Arm 2: placebo s.c. US	• Primary endpoints: safety and tolerability • Secondary endpoint: PK	• FPCD: Q4 2019 • Data anticipated: Q4 2021
Phase I **NCT04142424**	NASH/NAFLD F0-F3	60	MAD. 3 cohorts receiving AZD2693 and placebo in each cohort • Arm 1: AZD2693 s.c. • Arm 2: placebo s.c. US	• Primary endpoints: safety and tolerability • Secondary endpoint: PK	• FPCD: Q2 2021 • Data anticipated: H1 2022

AZD7503 (antisense oligonucleotide)

NASH

Trial	Population	Patients	Design	Endpoints	Status
Phase I	Healthy volunteers	64	SAD. 6 cohorts with 6 volunteers receiving AZD7503 and 2 volunteers receiving placebo in each cohort • Arm 1: AZD7503 s.c. • Arm 2: placebo s.c. US	• Primary endpoints: safety and tolerability • Secondary endpoint: PK	• FPCD: H2 2021 • Data anticipated: H2, 2022

AZD3427 (relaxin)

Heart failure

Trial	Population	Patients	Design	Endpoints	Status
Phase I **NCT04630067**	SAD – Healthy volunteers MAD – Heart failure	104	Multicentre single and multiple ascending dose study Part A SAD 6 cohorts • Arm 1: AZD3427 • Arm 2: placebo Part B MAD • Arm 1: AZD3427 • Arm 2: placebo US	• Primary endpoints: safety and tolerability	• FPCD: Q4 2020 • Data anticipated: H1 2022
Phase I **NCT04890548**	Heart failure	16	Mechanistic trial to evaluate the vasodilatory effects of AZD3427 • Cohort 1: HFpEF AZD3427 a sequence of 5 IA infusions into the brachial artery • Cohort 2: HFrEF a sequence of 5 IA infusions into the brachial artery UK	• Primary endpoint: change from baseline in absolute forearm blood flow • Secondary endpoints: change from baseline in forearm blood flow ratio in the infused arm and between arms; ADA; safety & tolerability	• FPCD: H2 2021 • Data anticipated: H1 2022

AZD3366

Cardiovascular disease

Trial	Population	Patients	Design	Endpoints	Status
Phase I **NCT04588727**	Healthy volunteers	103	SAD trial Part A 7 cohorts with 6 volunteers receiving AZD3366 and 2 volunteers receiving placebo in each cohort; three cohorts with Japanese subjects 5 receiving AZD3366 1 receiving placebo; 1 Chinese cohort of 6 receiving AZD3366 2 receiving placebo • Arm 1: AZD3366 • Arm 2: placebo Part B 12 subjects • Arm 1: AZD3366 + *Brilinta* + ASA • Arm 2: placebo + *Brilinta* + ASA	• Primary endpoints: safety and tolerability • Secondary endpoint: PK parameters	• FPCD: Q4 2020 • Data anticipated: H1 2022

Tozorakimab (IL33 ligand mAb)

Diabetic kidney disease

Trial	Population	Patients	Design	Endpoints	Status
Phase II **NCT04170543**	Adult patients with diabetic kidney disease	565	• Arm A: tozorakimab dose 1 + *Farxiga* • Arm B: tozorakimab dose 2 + *Farxiga* • Arm C: tozorakimab dose 3 + *Farxiga* • Arm D: tozorakimab dose 4 + *Farxiga* • Arm E: placebo + *Farxiga* USA, Canada, Japan and additional countries.	• Primary endpoints: safety and efficacy	• FPCD: Q4 2019 • Data anticipated: 2022+

AZD4831 (MPO inhibitor)

Cardiovascular disease

Trial	Population	Patients	Design	Endpoints	Status
Phase IIb/ Phase III **NCT04986202**	HFpEF	1485	Randomised, double blind • Arm 1: 2.5mg AZD4831 • Arm 2: 5mg AZD4831 • Arm 3: placebo Global trial	• Endpoints: Efficacy and Safety	• FPCD: Q3 2021 • Data anticipated: 2022+
Phase IIa **NCT03756285**	HFpEF	96	• Arm 1: AZD4831 • Arm 2: placebo Global trial – five countries	• Primary endpoint: The change from baseline in MPO activity in %	• FPCD: Q4 2018 • Data readout: Q2 2021
Phase I **NCT02712372**	Healthy patients	c. 96	SAD trial • Arm 1: AZD4831 • Arm 2: placebo Germany	• Primary endpoints: safety and tolerability • Secondary endpoint: PK parameters	• FPCD: Q3 2016 • LPCD: Q4 2016 • Data readout: Q2 2017
Phase I **NCT03136991**	Healthy patients	c. 40	MAD trial • Arm 1: AZD4831 • Arm 2: placebo USA	• Primary endpoints: safety and tolerability • Secondary endpoint: PK parameters	• FPCD: Q2 2017 • LPCD: Q4 2017 • Data readout: Q1 2018
Phase I **NCT04232345**	Healthy patients	32	MAD trial in Japanese and Chinese patients	• Primary endpoints: safety and tolerability	• FPCD Q1 2020 • Data readout: Q3 2021
Phase I **NCT04407091**	Healthy patients	6	Open label hADME trial • a single oral dose of [14C] AZD4831 UK	• Primary endpoints: mass balance, with routes and rates of elimination of [14C]AZD4831; Metabolite profiling and structural identification; PK and total radioactivity	• FPCD: Q2 2020 • LPCD: Q3 2020 • Data readout: Q3 2021
Phase I **NCT05052710**	Healthy patients	14	Open label • AZD4831 • AZD4831 and Midazolam UK	• Primary endpoints: PK parameters • Secondary endpoints: safety and tolerability	• FPCD: Q4 2021 • Data anticipated: H1 2022
Phase I **NCT04949438**	Renal Impairment	20	Open label • AZD4831	• Primary endpoints: PK parameters • Secondary endpoints: safety and tolerability	• FPCD: Q4 2021 • Data anticipated: 2022+

AZD5718 (FLAP inhibitor)

Cardiovascular disease & Chronic Kidney Disease

Trial	Population	Patients	Design	Endpoints	Status
Phase IIb NCT04492722	Proteinuric CKD	632	Randomised, double-blind, placebo-controlled, multicentre, dose-ranging trial • AZD5718 • placebo	• Primary endpoints: dose-response efficacy, safety, PK	• FPCD: Q4 2020 • Data anticipated: H2 2022
Phase IIa NCT03317002	CAD	129	• Arm 1: AZD5718 Dose A • Arm 2: AZD5718 Dose B • Arm 3: placebo Global trial – three countries in Europe	• Primary endpoint: PD effect of AZD5718 by assessment of u-LTE4	• FPCD: Q4 2017 • LPCD: Q4 2019 • Data readout: Q1 2021
Phase I NCT03948451	Healthy patients	6	Open label hADME trial • a single oral dose of ^{14}C-AZD5718 UK	• Primary endpoint: Mass balance, with routes and rates of elimination of ^{14}C-AZD5718; Metabolite profiling and structural identification; PK and total radioactivity	• FPCD: Q2 2019 • LPCD: Q2 2019
Phase I NCT04087187	Healthy patients	14	BA trial. Open-label, randomised, 3-period, 3-treatment, crossover design • AZD5718	• Primary endpoints: safety and tolerability, PK	• FPCD: Q4 2019 • LPCD: Q4 2019
Phase I NCT04210388	Healthy patients	12	BA trial. Open-label, randomised, single-dose, combined 2x2 dose and 3x3 dose crossover design in fixed sequence. • AZD5718	• Primary endpoints: bioavailability, safety and tolerability	• FPCD: Q1 2020 • LPCD: Q1 2020
Phase I NCT0473275	Healthy patients	16	BA trial. Open-label, randomised, single-dose, 3-period, single dose, crossover design • AZD5718	• Primary endpoints: bioavailability, safety and tolerability	• FPCD: Q1 2021 • LPCD: Q1 2021 • Data readout: Q4 2021

AZD8233 (PCSK9 inhibitor, subcutaneous)

Dyslipidaemia

Trial	Population	Patients	Design	Endpoints	Status
Phase II SOLANO NCT04964557	Dyslipidaemia	376	• Arm 1: AZD8233 dose • Arm 2: placebo 28 weeks 8 countries (US, Slovakia, Denmark, Poland, Spain, Netherlands, Czech Republic, Hungary)	• Primary endpoint: safety	• FPCD: Q3 2021 • LPCD: Q4 2021 • Data anticipated: H2 2022
Phase II **ETESIAN** **NCT04641299**	Dyslipidaemia	108	• Arm 1: High AZD8233 dose • Arm 2: Medium AZD8233 dose • Arm 3: Low AZD8233 dose • Arm 4: placebo 12 weeks 3 countries (US, Slovakia and Denmark)	• Primary endpoint: efficacy	• FPCD: Q4 2020 • LPCD: Q1 2021 • Data readout: Q3 2021
Phase II **HAYATE** **NCT04823611**	Dyslipidaemia	71	Part A: 11 subjects randomized in an 8:3 ratio • Arm 1: High AZD8233 dose • Arm 2: placebo Part B: 60 subjects randomized across three different treatment arms in a 1:1:1 ratio • Arm 1: Medium AZD8233 dose • Arm 2: Low AZD8233 dose • Arm 3: placebo 12 weeks Japan	• Part A primary endpoints: safety and tolerability • Part B primary endpoint: efficacy	• FPCD: Q1 2021 • Data anticipated: H2 2022
Phase I **NCT03593785**	Healthy subjects	72	SAD trial 7 cohorts • Arm1: AZD8233 • Arm 2: placebo US	• Primary endpoints: safety and tolerability • Secondary endpoints: PK and PD parameters	• FPCD: Q3 2018 • LPCD: Q3 2019 • Data readout: Q2 2021
Phase I **NCT04155645**	Dyslipidaemia	33	MAD trial 3 cohorts • Arm1: AZD8233 • Arm 2: placebo US	• Primary endpoints: safety and tolerability • Secondary endpoints: PK and PD parameters	• FPCD: Q1 2020 • LPCD: Q4 2020 • Data readout: Q3 2021

MEDI8367

Chronic kidney disease

Trial	Population	Patients	Design	Endpoints	Status
Phase I **NCT04365218**	Healthy volunteers CKD	70	SAD trial 6 cohorts • Arm 1: MEDI8367 s.c. • Arm 2: placebo s.c. US	• Primary endpoints: safety and tolerability • Secondary endpoints: PK parameters, ADA	• FPCD: Q3 2020

AZD8601 (VEGF-A modified RNA)

Cardiovascular disease

Trial	Population	Patients	Design	Endpoints	Status
Phase IIa **NCTT03370887**	HF	Up to 33	• Arm 1: AZD8601 Dose A • Arm 2: AZD8601 Dose B • Arm 3: placebo Finland, Germany	• Primary endpoints: safety and tolerability	• FPCD: Q1 2018 • Data readout: Q3 2021
Phase I **NCT02935712**	Type-2 diabetic patients	c. 60	SAD trial • Arm 1: AZD8601 • Arm 2: placebo Germany	• Primary endpoints: safety and tolerability	• FPCD: Q1 2017 • LPCD: Q3 2017 • Data readout: Q1 2018

AZD9977 (MCR modulator)

Heart failure

Trial	Population	Patients	Design	Endpoints	Status
Phase IIb **NCT04595370**	Heart failure with chronic kidney disease	540	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 15 mg + *Farxiga* 10 mg • Arm 2: AZD9977 50 mg + *Farxiga* 10 mg • Arm 3: AZD9977 150 mg + *Farxiga* 10 mg • Arm 4: AZD9977 150 mg • Arm 5: *Farxiga* 10 mg • Arm 6: placebo 12 weeks Trial conducted in 20 countries globally	• 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 placebo, AZD9977 alone *Farxiga* and 3 doses of AZD9977 combined with *Farxiga* on UACR; safety, tolerability and serum potassium values; eGFR	• FPCD: Q2 2021 • Data anticipated: H2 2022
Phase I **NCT03435276**	Healthy volunteers	27	MAD trial 3 cohorts • Arm 1: AZD9977 • Arm 2: placebo UK.	• Primary endpoints: safety and tolerability • Secondary endpoints: PK parameters	• FPCD: Q1 2018 • LPCD: Q2 2018 • Data readout: Q3 2018
Phase I **NCT03450759**	Healthy volunteers	12	BA trial of four different oral formulations of AZD9977 and influence of food UK.	• Primary endpoints: relative bioavailability vs. oral suspension (reference) • Secondary endpoints: PK parameters	• FPCD: Q2 2018 • LPCD: Q2 2018 • Data readout: Q3 2018
Phase I **NCT03682497**	HF	60	• Arm 1: AZD9977 • Arm 2: spironolactone	• Primary endpoint: serum potassium	• FPCD: Q4 2018 • LPCD: Q1 2019
Phase I **NCT03843060**	Healthy volunteers	14	DDI trial • Arm 1: AZD9977 • Arm 2: AZD9977 + itraconazole US	• Primary endpoints: PK parameters • Secondary endpoints: safety and tolerability	• FPCD: Q1 2019 • LPCD: Q1 2019 • Data readout: Q3 2019

AZD9977 (MCR modulator)

Heart failure

Trial	Population	Patients	Design	Endpoints	Status
Phase I **NCT03801967**	Healthy volunteers	45	JSMAD trial Single and multiple-ascending dose administration in Japanese healthy volunteers. UK	• Primary endpoints: safety and tolerability • Secondary endpoints: PK parameters	• FPCD: Q1 2019 • LPCD: Q2 2019 • Data readout: Q3 2019
Phase I **NCT03804645**	Healthy volunteers	12	BA trial Investigation of four different oral formulations of AZD9977 and influence of food. UK	• Primary endpoints: relative bioavailability vs. capsule formulation (reference); PK parameters	• FPCD: Q1 2019 • LPCD: Q2 2019 • Data readout: Q3 2019
Phase I **NCT04469907**	Renal impairment	32	• AZD9977 US	• Primary endpoints: PK parameters • Secondary endpoints: safety and tolerability	• FPCD: Q3 2020 • Data anticipated: Q4 2021
Phase I **NCT04686591**	Healthy volunteers	8	hADME trial • single oral dose ^{14}C-AZD9977 UK	• Primary endpoints: absolute bioavailability, the mass balance, rates and routes of elimination • Secondary endpoints: safety and tolerability	• FPCD: Q1 2021 • LPCD: Q1 2021 • Data anticipated: Q4 2021
Phase I **NCT04798222**	Healthy volunteers	20	Rel BA trial Study to Assess the Bioavailability of Different Formulations of AZD9977 and Dapagliflozin and Influence of Food in Selected Formulations Germany	• Primary endpoints: relative bioavailability of 9977 and Dapagliflozin in different capsule formulations; PK parameters	• FPCD: Q3 2021 • LPCD: Q3 2021 • Data anticipated: Q4 2021

Zibotentan (endothelin receptor antagonist)

Chronic kidney disease

Trial	Population	Patients	Design	Endpoints	Status
Phase IIb **ZENITH-CKD** **NCT04724837**	CKD	660	Part A: 132 participants equally randomised across 4 arms: • Arm 1: zibotentan dose A + *Farxiga* 10 mg once daily • Arm 2: zibotentan dose A once daily • Arm 3: *Farxiga* 10 mg once daily • Arm 4: placebo once daily Part B: 528 participants equally randomised across 6 arms: • Arm 1: zibotentan dose C + *Farxiga* 10 mg once daily • Arm 2: zibotentan dose B + *Farxiga* 10 mg once daily • Arm 3: zibotentan dose A + *Farxiga* 10 mg once daily • Arm 4: zibotentan dose A once daily • Arm 5: *Farxiga* 10 mg once daily • Arm 6: placebo once daily Global	• Primary endpoint: change in log-transformed UACR from baseline to week 12. • Secondary endpoints: change in log-transformed UACR from baseline to week 12; change in blood pressure from baseline (Visit 2) to week 12; least squares mean change of UACR at week 12 from the 3 Zibo/Dapa dose groups and the *Farxiga* monotherapy group; change in eGFR from baseline to week 1, week 12 and week 14; change in eGFR from week 1 to week 12.	• FPCD: Q2 2021 • Data anticipated: H2 2022
Phase 1 **NCT04991571**	Healthy volunteers	28	• Part 1 of the study is intended to collect samples for Metabolites in Safety Testing (MIST) analysis after administration of multiple doses of zibotentan. • Part 2 of the study is designed to evaluate the relative bioavailability of zibotentan and dapagliflozin after dosing with two different fixed-dose combination (FDC) formulations and dosing with separate formulations of zibotentan and dapagliflozin	Part 1: Metabolites in Safety Testing MIST analysis. No PK statistical analysis will be performed. Part 2: PK parameters	• FPCD: Q3 2021 • Data anticipated: Q1 2022

AZD5462 (relaxin)

Heart failure

Trial	Population	Patients	Design	Endpoints	Status
Phase I NCT04994106	SAD – Healthy volunteers MAD – Healthy volunteers		Single centre single and multiple ascending dose study Part A SAD 8 cohorts • Arm 1: AZD5462 • Arm 2: placebo Part B MAD 5 cohorts • Arm 1: AZD5462 • Arm 2: placebo US	• Primary endpoints: safety and tolerability	• FPCD: Q4 2021 • Data anticipated: H1 2022

MEDI6570

Cardiovascular

Trial	Population	Patients	Design	Endpoints	Status
Phase IIb NCT04610892	Post MI	792	Evaluation of anti-inflammatory potential and effect on surrogates for atherosclerotic and heart failure (HF) events • Arm 1: High MEDI6570 dose • Arm 2: Medium MEDI6570 dose • Arm 3: Low MEDI6570 dose • Arm 4: placebo US, Canada, Hungary, Japan, Czech Republic, Italy, Spain, Netherlands, Poland, UK, Australia, Russia	• Primary endpoints: efficacy and safety	• FPCD: Q4 2020 • Data anticipated: 2022+
Phase I NCT03654313	Atherosclerotic cardiovascular disease	88	SAD followed by MAD trial • MEDI6570	• Primary endpoints: safety and tolerability	• FPCD: Q4 2018 • LPCD: Q3 2020 • Data readout: Q3 2021

AZD0449 (inhaled JAK-1 inhibitor)

Asthma

Trial	Population	Patients	Design	Endpoints	Status
Phase I **NCT03766399**	Healthy subjects and patients with mild asthma	131	SAD/MAD/Bridge trial Part 1 SAD • Arm 1: AZD0449 (nebulised) • Arm 2: placebo (nebulised) Part 2 MAD • Arm 1: AZD0449 (nebulised) • Arm 2: placebo (nebulised) Part 3 bridge • Arm 1: AZD0449 (DPI) • Arm 2: placebo (DPI) UK and NZ	• Primary endpoints: safety and tolerability • Secondary endpoints: PK parameters, FENO	• FPCD: Q4 2018 • LPCD: Q2 2021 • Data anticipated: Q4 2021

AZD4604 (inhaled JAK-1 inhibitor)

Asthma

Trial	Population	Patients	Design	Endpoints	Status
Phase I **NCT04769869**	Healthy subjects and patients with mild asthma	137	SAD/MAD/POM trial Part 1 SAD • Arm 1: AZD0449 (DPI) • Arm 2: placebo (DPI) Part 2 MAD • Arm 1: AZD0449 (DPI) • Arm 2: placebo (DPI) Part 3 POM • Arm 1: AZD0449 (DPI) • Arm 2: placebo (DPI) UK	• Primary endpoints: safety and tolerability • Secondary endpoints: PK parameters, FENO	• FPCD: Q4 2021 • Data anticipated: 2022+

AZD1402 (IL4 receptor alpha antagonist)

Asthma

Trial	Population	Patients	Design	Endpoints	Status
Phase IIa **NCT04643158**	Patients with asthma on medium dose inhaled corticosteroids	405	Randomised, placebo-controlled, double-blinded, multicentre, 2-part trial. Part 1 population with asthma controlled on medium dose ICS-LABA Part 1a • Arm 1: AZD1402 Dose 1 (low) (DPI) • Arm 2: AZD1402 Dose 2 (DPI) • Arm 3: placebo Part 1b • Arm 1: AZD1402 Dose 3 (high) (DPI) • Arm 2: placebo Part 2 population uncontrolled on medium dose ICS-LABA • Arm 1: AZD1402 Dose 1 (DPI) • Arm 2: AZD1402 Dose 2 (DPI) • Arm 3: AZD1402 Dose 3 (DPI) • Arm 4: placebo Ukraine, Australia, Germany	• Part 1 primary endpoints: safety and tolerability, PK • Part 2 primary endpoint: change in FEV1	• FPCD: Q2 2021 • Data anticipated: H2 2022
Phase Ib **NCT03574805** **Partnered**	Patients with mild asthma	84	PoM trial. A dose-escalating, single blind trial of multiple doses of PRS-060 administered by oral Inhalation In subjects with mild asthma Australia	• Primary endpoints: safety and tolerability • Secondary endpoints: PK parameters, potential immunogenicity, change in FENO	• FPCD: Q3 2018 • LPCD: Q3 2020 • Data readout: Q4 2020

Tozorakimab (IL33 ligand mAb)

COPD, atopic dermatitis, asthma

Trial	Population	Patients	Design	Endpoints	Status
Phase II **NCT04212169**	Adult subjects with atopic dermatitis	152	Randomised, blinded, placebo-controlled trial • Arm 1: tozorakimab s.c. • Arm 2: tozorakimab s.c. • Arm 3: tozorakimab s.c. • Arm 4: placebo s.c. US, Australia, Germany, Poland, UK & Spain	• Primary endpoint: change from baseline at week 16 in Eczema Area and Severity Index (EASI) score • Secondary endpoints: safety and other efficacy measures	• FPCD: Q4 2019 • Data anticipated: H1 2022
Phase II **NCT04570657**	Adult participants with uncontrolled moderate to severe asthma	278	Randomised, double-blind, placebo-controlled trial • Arm 1: tozorakimab Dose 1 s.c. • Arm 2: tozorakimab Dose 2 s.c. • ARM 2: placebo s.c. 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 • Data anticipated: H2 2022
Phase II **NCT04631016**	Adult subjects COPD and chronic bronchitis	322	Randomised, double-blind, placebo-controlled, parallel group, proof of concept trial • Arm 1: tozorakimab s.c. • ARM 2: placebo s.c. US, Australia, Canada, Czech Republic, Denmark, Germany, Hungary, Israel, Netherlands, New Zealand, Poland, South Africa, Spain, Taiwan and UK	• Primary endpoint: change from baseline at week 12 in FEV1 • Secondary endpoints: safety and other efficacy measures	• FPCD: Q1 2021 • Data anticipated: 2022+
Phase I **NCT05070312**	Healthy subjects	36	Randomized, double-blind, placebo-controlled, doseascending trial • Arm 1: tozorakimab Dose 1 s.c. • Arm 2: placebo s.c. • Arm 3: tozorakimab Dose 2 s.c. • Arm 4: placebo s.c. China	• Primary endpoint: to characterize the pharmacokinetics of tozorakimab • Secondary endpoints: to evaluate the immunogenicity of tozorakimab	• FPCD: Q3 2021 • Data anticipated: 2022+

MEDI0618 (PAR2 antagonist mAb)

Osteoarthritis pain

Trial	Population	Patients	Design	Endpoints	Status
Phase I NCT04198558	Healthy volunteers	64	SAD trial • Arm 1: MEDI0618 i.v. • Arm 2: placebo i.v. • Arm 3: MEDI0618 s.c • Arm 4: placebo s.c Europe only	• Primary endpoints: safety and tolerability • Secondary endpoint: PK	• FPCD: Q4 2019 • Data anticipated: H1 2022

MEDI1341 (alpha-synuclein mAb)

Parkinson's disease

Trial	Population	Patients	Design	Endpoints	Status
Phase I NCT03272165	Healthy volunteers	48	SAD trial • Arm 1: MEDI1341 i.v. • Arm 2: placebo i.v. US only	• Primary endpoints: safety and tolerability • Secondary endpoints: PK, PD	• FPCD: Q4 2017 • LPCD: Q4 2020 • Data anticipated: Q4 2021
Phase I NCT04449484	Parkinson's Disease	36	MAD trial • Arm 1: MEDI1341 i.v. • Arm 2: placebo i.v. US only	• Primary endpoints: safety and tolerability • Secondary endpoints: PK, PD	• FPCD: Q3 2020 • Data anticipated: 2022+

AZD4041 (orexin 1 receptor antagonist)

Opioid use disorder

Trial	Population	Patients	Design	Endpoints	Status
Phase I NCT04076540 Partnered with Eolas Therapeutics Inc and NIH	Healthy volunteers	48	Randomised, double blind, SAD trial • Arm 1: AZD4041 • Arm 2: placebo US only	• Primary endpoints: safety and tolerability • Secondary endpoints: PK, PD	• FPCD: Q4 2019 • Data anticipated: Q4 2021

MEDI7352 (NGF TNF bispecific mAb)

Osteoarthritis pain

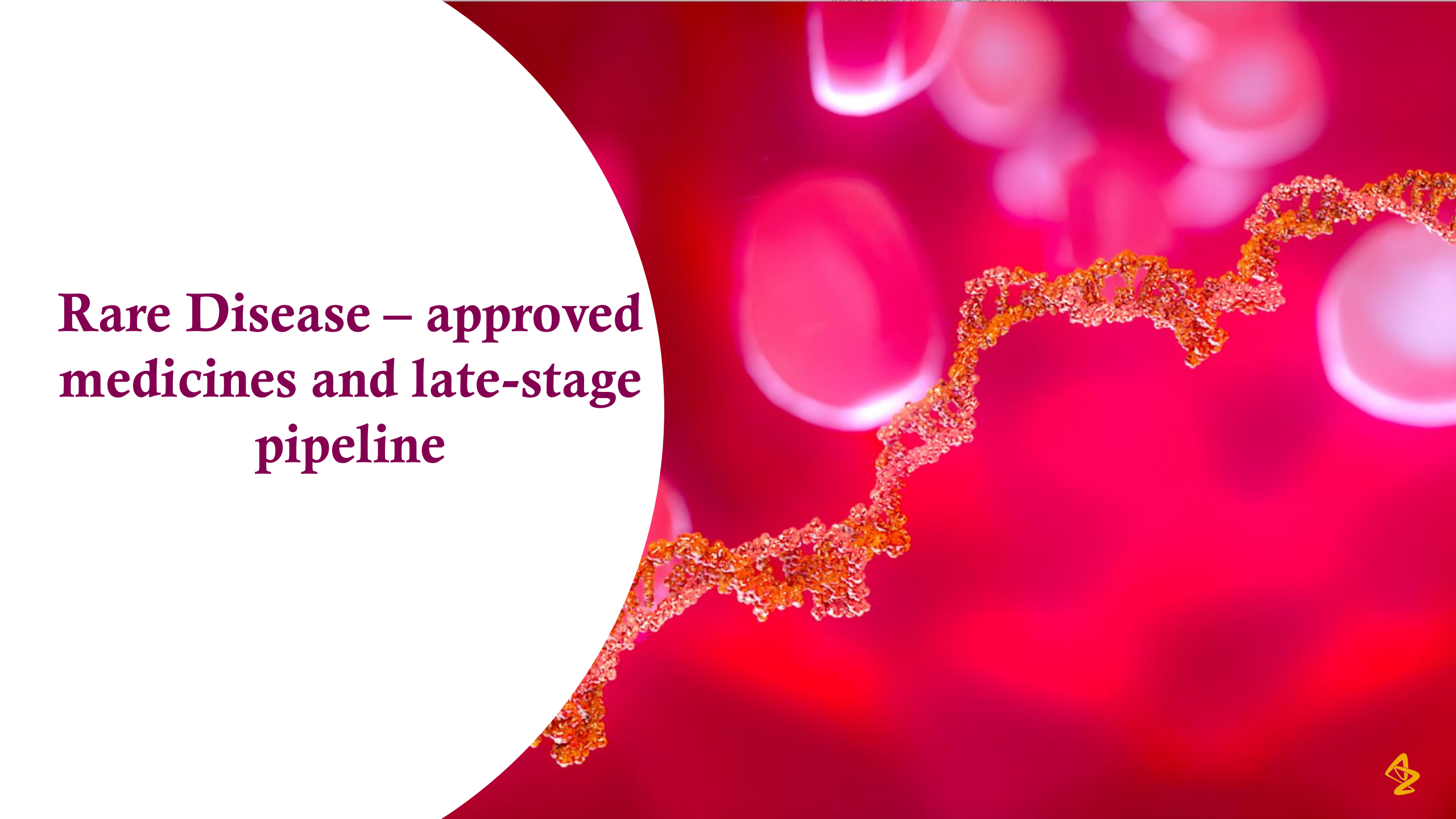
Trial	Population	Patients	Design	Endpoints	Status
Phase IIb **NCT04675034**	Painful osteoarthritis of the knee	300	MAD trial • Arm 1: MEDI7352 s.c • Arm 2: placebo s.c Global (8 countries)	• Primary endpoint: dose response • Secondary endpoints: safety, tolerability, PK, PD, ADA	• FPCD: Q1 2021 • Data anticipated: H2 2022
Phase II **NCT03755934**	Painful diabetic neuropathy	271	MAD trial • Arm 1: MEDI7352 i.v. • Arm 2: placebo i.v. Europe only	• Primary endpoint: dose response • Secondary endpoints: safety, tolerability, PK, PD	• FPCD: Q4 2018 • Data anticipated: H2 2022
Phase I **NCT02508155**	Painful osteoarthritis of the knee	160	SAD & MAD trial • Arm 1: MEDI7352 i.v. • Arm 2: placebo i.v. • Arm 3: MEDI7352 s.c • Arm 4: placebo s.c Europe only	• Primary endpoints: safety and tolerability • Secondary endpoints: PK, PD	• FPCD: Q1 2016 • LPCD: Q4 2020 • Data readout: Q2 2021
Phase I **NCT04770428**	Healthy volunteers Japanese and Caucasian	20	MAD trial • Arm 1: MEDI7352 s.c • Arm 2: placebo s.c Europe only	• Primary endpoints: safety and tolerability • Secondary endpoints: PK, PD, ADA	• FPCD: Q2 2021 • Data anticipated: Q4 2021

MEDI3902 (Anti-Pseudomonas A mAb)

Infections

Trial	Population	Patients	Design	Endpoints	Status
Phase II NCT02696902	Intubated ICU	195	SAD trial • Arm 1: MEDI3902 i.v. • Arm 2: placebo i.v.	• Primary endpoints: safety and efficacy	• FPCD: Q2 2016 • Data readout: Q4 2020

Rare Disease – approved medicines and late-stage pipeline



Soliris (anti-complement C5 mAb)

Neurology

Trial	Population	Patients	Design	Endpoints	Status
Phase III **ECU-GBS-301** **NCT04752566**	Guillain Barré syndrome	57	• Arm 1: *Soliris* i.v. once weekly for 4 weeks • Arm 2: Placebo Japan-only	• Primary endpoint: Time to first reaching a Hughes functional grading scale score ≤ 1	• FPCD: Q1 2021 • Data anticipated: H2 2022

Ultomiris (anti-complement C5 mAb)

Haematology & nephrology

Trial	Population	Patients	Design	Endpoints	Status
Phase III **ALXN-1210-PNH-303** **NCT03748823**	PNH and aHUS	136	• *Ultomiris* s.c.	• Primary endpoint: Day 71 serum *Ultomiris* C_{trough}	• FPCD: Q1 2019 • Data readout: Q2 2020 • Primary endpoint met
Phase III **ALXN-1210-TMA-315** **NCT04743804**	Thrombotic microangiopathy associated with a trigger	100	• Arm 1: *Ultomiris* Q8W • Arm 2: Placebo	• Primary endpoint: Complete TMA response • Secondary endpoints: Time to complete TMA response, Hematologic response, Renal response	• FPCD: Q3 2021 • Data anticipated: 2022+
Phase III **ALXN-1210-TM-313** **NCT04543591**	Thrombotic microangiopathy associated with haematopoietic stem cell transplant	184	• Arm 1: *Ultomiris* Q8W • Arm 2: Placebo	• Primary endpoint: TMA response • Secondary endpoints: Time to TMA response, TMA relapse	• FPCD: Q4 2020 • Data anticipated: 2022+
Phase III **ALXN-1210-TM-314** **NCT04557735**	Paedeatric thrombotic microangiopathy associated with haematopoietic stem cell transplant	40	• Arm 1: *Ultomiris* administered once every 4-8 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: 2022+
Phase II **ALXN-1210-NEPH-202** **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: Both cohorts, percentage change in proteinuria from baseline to Week 26 • Secondary endpoints: Both cohorts, percentage change in proteinuria from baseline to Week 50	• FPCD: Q1 2021 • Data anticipated: 2022+

Ultomiris (anti-complement C5 mAb)

Neurology

Trial	Population	Patients	Design	Endpoints	Status
Phase II/III **ALXN-1210-ALS-308** **NCT04248465**	Amyotrophic lateral sclerosis	382	• Arm 1: *Ultomiris* • Arm 2: placebo	• Primary endpoint: change from baseline in ALSFRS-R total score	• Trial halted post futility analysis Q3 2021
Phase III **ALXN-1210-NMO-307** **NCT04201262**	Neuromyelitis optica spectrum disorder	58	• Arm 1: *Ultomiris* Q8W	• Primary endpoint: time to first adjudicated on-trial relapse	• FPCD: Q4 2019 • Data anticipated: H1 2022
Phase III **ALXN-1210-MG-306** **NCT03920293**	Generalised myasthenia gravis	175	• Arm 1: *Ultomiris* • Arm 2: placebo	• Primary endpoint: change from baseline in MG-ADL total score at Week 26	• Data readout: Q2 2021 • Primary endpoint met

ALXN1840 (bis-choline tetrathiomolybdate)

Wilson disease

Trial	Population	Patients	Design	Endpoints	Status
Phase III WTX101-301 FoCus NCT03403205	Wilson Disease	215	• Arm 1: ALXN1840 was administered orally for 48 weeks at doses ranging from 15 milligrams (mg) every other day (QOD) up to a titrated dose of 60 mg daily. • Arm 2: Standard of care	• Primary endpoint: daily mean AUEC of dNCC • Secondary endpoint: change from baseline in the UWDRS Part II total score	• Data readout: Q3 2021 • Primary endpoint met
Phase II ALXN1840-WD-204 NCT04573309	Wilson Disease	10	• Arm 1: Participants will be administered ALXN1840 at a dose of 15 milligrams (mg)/day on Day 1 through Day 28 and then increased to 30 mg/day on Day 29 through Day 39	• Primary endpoint: mean daily copper balance	• FPCD: Q3 2020 • Data anticipated: H2 2022
Phase II ALXN1840-WD-205 NCT04422431	Wilson Disease	31	• Arm 1: ALXN1840, Participants will be initiated at 15 milligrams once daily, then the dose will be increased to 30 milligrams once daily at Week 6.	• Primary endpoint: change from baseline at Week 48 in liver copper concentration	• FPCD: Q4 2020 • Data anticipated: H2 2022

Andexxa (anti-factor Xa reversal)

Haematology

Trial	Population	Patients	Design	Endpoints	Status
Phase II **19-515** **NCT04233073**	Urgent surgery	100	• 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 • Data anticipated: 2022+

CAEL-101 (fibril-reactive mAb)

AL amyloidosis

Trial	Population	Patients	Design	Endpoints	Status
Phase III **CAEL101-302** **NCT04512235**	Mayo stage IIIa amyloidosis	267	• Arm 1: CAEL-101 combined with SoC for plasma cell dyscrasia (PCD) • Arm 2: placebo combined with SoC for PCD	• Primary: time from first dose of trial drug until death or end of trial • Secondary: change in distance walked during a six-minute walk test, and quality of life measures	• FPCD: Q4 2020 • Data anticipated: 2022+
Phase III **CAEL101-301** **NCT04504825**	Mayo stage IIIb amyloidosis	110	• Arm 1: CAEL-101 combined with SoC for PCD • Arm 2: placebo combined with SoC for PCD	• Primary: time from first dose of trial drug until death or end of trial • Secondary: change in distance walked during a six-minute walk test, and quality of life measures	• FPCD: Q1 2021 • Data anticipated: 2022+
Phase II **CAEL101-203** **NCT04304144**	Mayo Stage I, Stage II and Stage IIIa amyloidosis	25	• Arm 1: CAEL-101 combined with SoC CyBorD • Arm 2: placebo combined with SoC CyBorD and daratumumab	• Primary: dose toxicity – occurrence of dose limiting toxicity (DLT) during the first 4 weeks of therapy • Secondary: area under the plasma curve concentration versus time curve (AUC)	• FPCD: Q1 2020 • Data anticipated: 2022+

acoramidis (ALXN2060)

ATTR-CM

Trial	Population	Patients	Design	Endpoints	Status
Phase III **ALXN2060-TAC-302** **NCT04622046**	ATTR-CM	22	• Arm 1: 800 mg acoramidis (ALXN2060) administered twice daily	• Primary endpoint: Change from Baseline to Month 12 of Treatment In Distance Walked During the Six-minute Walk Test, All-cause mortality and cardiovascular-related hospitalization over a 30-month period	• FPCD: Q4 2020 • Data anticipated: H2 2022

danicopan (ALXN2040 Factor D inhibitor)

Haematology & ophthalmology

Trial	Population	Patients	Design	Endpoints	Status
Phase III **ALXN2040-PNH-301** **NCT04469465**	PNH with clinically relevant extravascular haemolysis	84	• Arm 1: danicopan (ALXN2040) + 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 anticipated: H2 2022
Phase II **ALXN2040-GA-201** **NCT05019521**	Geographic atrophy	330	• Arms 1-3: danicopan (ALXN2040) dosed at 100-400mg daily • 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: 2022+

Rare Disease – early-stage development

ALXN1720 (anti-C5 bi-specific minibody)

Neurology

Trial	Population	Patients	Design	Endpoints	Status
Phase I ALXN1720-HV-101 NCT04920370	Healthy Volunteers	96	• Single & Multiple Ascending Dosing of ALXN1720 via s.c. and i.v. administration • Placebo	• Primary: Incidence of TEAEs and SAEs	• FPCD: Q3 2019 • Data anticipated: H1 2022

ALXN1830 (anti-FcRn mAb)

Haematology & neurology

Trial	Population	Patients	Design	Endpoints	Status
Phase I **ALXN1830-HV-108** **NCT04730804**	Healthy Volunteers	48	• Single and multiple ascending dose cohorts of ALXN1830 • Placebo	• Primary endpoint: Number of participants with TEAEs	• FPCD: Q1 2021 • Paused

ALXN2050 (factor D inhibitor)

Haematology, nephrology, neurology

Trial	Population	Patients	Design	Endpoints	Status
Phase II **ACH228-110** **NCT04170023**	PNH	26	• Arm 1: ALXN2050 Monotherapy, with groups including treatment-naïve, C5 inhibitor treatment experienced, and patients previously receiving danicopan (ALXN2040)	• Primary endpoint: Change in haemoglobin relative to baseline • Secondary endpoints: Number of participants who have transfusion avoidance, Change in lactate dehydrogenase relative to baseline	• FPCD: Q4 2019 • Data anticipated: 2022+

ALXN1820 (anti-properdin)

Haematology

Trial	Population	Patients	Design	Endpoints	Status
Phase I **ALXN1820-HV-101** **NCT04631562**	Healthy Volunteers	80	• Arm 1: ALXN1820 administered s.c. or i.v., multiple ascending doses • Arm 2: Placebo	• Primary: Participants with TEAEs	• FPCD: Q1 2021 • Data anticipated: H2 2022

ALXN1850 (next generation asfotase alfa)

Hypophosphatasia

Trial	Population	Patients	Design	Endpoints	Status
Phase 1 ALXN1850-HPP-101 NCT04980248	HPP	15	• Arm 1: ALXN1850, 3 cohorts at low, medium, and high dosages	• Primary: Incidence of TEAEs and TESAEs	• FPCD: Q3 2021 • Data anticipated: H2 2022

List of abbreviations

14C	Radioactive isotope of carbon, Carbon 14
1L, 2L, 3L	1st, 2nd or 3rd line
5-FU	5-fluorouracil
A2AR	Adenosine A2A receptor
ACQ	Asthma control questionnaire
ACR	American college of rheumatology response scoring system
ADA	Anti-drug antibodies
ADC	Antibody-drug conjugate
ADP	Adenosine diphosphate
AE	Adverse event
aHUS	Atypical haemolytic uraemic syndrome
AI	Auto-injector
AKT	Protein kinase B
ALK	Anaplastic large-cell lymphoma kinase
ALSFRS-R	Amyotrophic Lateral Sclerosis Functional Rating Scale-Revised
APFS	Accessorised pre-filled syringe
AQLQ	Asthma quality of life questionnaire
AS	Albuterol sulphate
ATM	Ataxia-telangiectasia mutated kinase
ATR	Ataxia telangiectasia and rad3-related protein
ATTR-CM	Transthyretin amyloid cardiomyopathy
AUC	Area under curve
AUEC	Area under the effect-time curve
B7RP	B7-related protein-1
BA	Bioavailability
BAFF	B-cell activating factor
BCG	Bacillus Calmette–Guérin
BCMA	B-cell maturation antigen
BDA	Budesonide albuterol
BFF	Budesonide and formoterol fumarate
BGF	Budesonide, glycopyrronium and formoterol fumarate
BICR	Blinded independent central review
BID	Bis in die (twice per day)
BIG	Big ten cancer research consortium
BMD	Bone mineral density
BMFI	Bone metastasis-free interval
BMI	Body mass index
BRCAwt	Breast cancer wild-type gene
BRD4	Bromodomain-containing protein 4
BTC	Biliary tract carcinoma
BTK	Bruton's tyrosine kinase
CA-125	Cancer antigen 125
CAD	Coronary artery disease
CBR	Clinical benefit rate
CD	Cluster of differentiation
CDK	Cyclin-dependent kinase
CE	Clinically evaluable
CHD	Coronary heart disease
Chemo	Chemotherapy
CHF	Chronic heart failure
CKD	Chronic kidney disease
CLL	Chronic lymphocytic leukaemia
CMAX	Maximum observed plasma concentration
C-MET	Tyrosine-protein kinase Met
CNS	Central nervous system
COPD	Chronic obstructive pulmonary disease
CR	Complete response
CRC	Colorectal cancer
CrCl	Creatinine clearance
CRR	Complete response rate
CTC	Circulating tumour cell
CTLA-4	Cytotoxic T-lymphocyte–associated antigen 4
CV	Cardiovascular
CVOT	Cardiovascular outcomes trial
CVRM	Cardiovascular renal and metabolism
CXCR2	C-X-C Motif chemokine receptor 2
DB	Double blind
DC	Disease control
DCR	Disease control rate
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
DoCR	Durability of complete response
dNCC	Directly Measured Non-ceruloplasmin-bound Copper
DoR	Duration of response
DPI	Dry powder inhaler
DRFI	Disease recurrence-free interval
DXA	Dual energy X-ray absorptiometry
EBRT	External beam radiation therapy
ECG	Electrocardiogram
EFS	Event-free survival
eGFR	Estimated glomerular filtration rate
EGFR	Epidermal growth factor receptor
ER	Oestrogen receptor
ERK	Extracellular signal-regulated kinase
ESR	Externally sponsored trial
ESR1	Oestrogen receptor 1
ESSC	Esophageal squamous cell carcinoma
ET	Endocrine therapy
FAF	Fundus Autofluorescence
FDC	Fixed-dose combination
FeNO	Fractional nitric oxide concentration in exhaled breath
FEV	Forced-expiratory volume
FGFR	Fibroblast growth factor receptor
FLAP	5-lipoxygenase-activating protein
FPDC	First patient commenced dosing
FPG	Fasting plasma glucose
GA	Gestational age
GA	Geographic atrophy
GBM	Glioblastoma
gBRCAm or tBRCAm	Germline or tumour (somatic) BRCA mutation
GEJ	Gastric/gastro-oesophogeal junction
GFF	Glycopyrronium and formoterol fumarate
GLP-1	Glucagon-like peptide-1
GMFRs	Geometric mean fold rises
GMTs	Geometric mean titers
hADME	Human mass balance
HAI	Haemagglutination-inhibition
HbA1c	Haemoglobin A1c
HCC	Hepatocellular carcinoma
HD	High dose
HDL-C	High-density lipoprotein cholesterol
HER2	Human epidermal growth factor receptor 2
HF	Heart failure
HFpEF	Heart failure with preserved ejection fraction

List of abbreviations

HFrEF	Heart failure with reduced ejection fraction
HGFR	Met/hepatocyte growth factor receptor
HGSC	High grade serous carcinoma
hHF	Hospitalisation for heart failure
HIF-PHI	Hypoxia inducible factor - prolyl hydroxylase inhibitor
HNSCC	Head and neck squamous-cell carcinoma
HPV	Human papillomavirus
HRD	Homologous recombination deficiency
HRRm	Homologous recombination repair mutation
i	inhibitor
IA	Investigator-assessed
ICS	Inhaled corticosteroid
ICU	Intensive care unit
IDFS	Invasive disease-free survival
IgAN	Immunoglobulin A nephropathy
IL	Interleukin
i.m.	Intramuscular
IRC	Independent review committee
ISS	Investigator-sponsored studies
i.v.	Intravenous
J-SD	Japanese single dose
Ki67	Protein that is encoded by the MKI67 gene in human
LAAB	Long-acting antibody
LABA	Long -acting beta agonist
LAMA	Long-acting muscarinic agonist
LCAT	Lecithin-cholesterol acyltransferase
LCM	Lifecycle management
LDH	Lactate dehydrogenase
LN	Lupus nephritis
LOCS III	Lens opacities classification system III
LPCD	Last patient commenced dosing
LV	Left ventricle
m	Mutation
mAb	Monoclonal antibody
MABA	Muscarinic antagonist-beta2 agonist
MACE	Major adverse cardiac events
MAD	Multiple ascending dose
MCC	Mucociliary clearance
MCL	Mantle cell lymphoma
MCL1	Myeloid leukemia cell differentiation protein 1
mCRPC	Metastatic castrate-resistant prostate cancer
MD	Medium dose
MDI	Metered-dose inhaler
MDS	Myelodysplastic syndrome
MEK	Mitogen-activated protein kinase
MET	Tyrosine-protein kinase Met
MG-ADL	Myasthenia Gravis-Activities Of Daily Living
MI	Myocardial infarction
MMT	Mixed meal test
MPO	Myeloperoxidase
mPR	Major pathological response
MRI	Magnetic resonance imaging
MTD	Maximum tolerated dose
NaC	Sodium channel
NCI	National cancer institute (US)
NCPV	Noncalcified plaque volume
NF1	Neurofibromatosis type 1
NGF	Nerve growth factor
NHL	Non-Hodgkin's lymphoma
NIH	National Institute of Health (US)
NKG2a	Natural killer cell C-type lectin receptor G2A
NME	New molecular entity
NRG	National clinical trials network in oncology (US)
NSCLC	Non-small cell lung cancer
OCS	Oral corticosteroid
OD	Once daily
OGTT	Oral glucose tolerance test
OR	Objective response
ORR	Objective response rate
OS	Overall survival
PARP	Poly ADP ribose polymerase
PASI	Psoriasis area severity index
PBD	Pyrrolobenzodiazepine
pCR	Pathological complete response
PD	Pharmacodynamics
PD-1	Programmed cell death protein 1
PDAC	Pancreatic ductal adenocarcinoma
PDE4	Phosphodiesterase type 4
PD-L1	Programmed death-ligand 1
PET	Positron-emission tomography
PFS	Progression free survival
PgR	Progesterone receptor
PI3K	Phosphoinositide 3-kinase
PIK3CA	Phosphatidylinositol 3 kinase catalytic alpha gene
PK	Pharmacokinetics
PLL	Prolymphocytic leukaemia
pMDI	Pressurised metered dose inhaler
PN	Plexiform neurofibromas
PNH	Paroxysmal nocturnal haemoglobinuria
POC	Proof of concept
POM	Proof of mechanism
pPCI	Primary percutaneous coronary intervention
PR	Partial response
pre-BD	Pre-bronchodilator
PRO	Patient reported outcome
PRR	Recurrent platinum resistant
PS	Propensity score
PSA	Prostate-specific antigen
PSC	Pulmonary sarcomatoid carcinoma
PSMA	Prostate-specific membrane antigen
PTEN	Phosphatase and tensin homolog gene
Q2,3,4,8W	Quaque (every) two, three… weeks
QD	Quaque in die (once a day)
QID	Quarter in die (four times a day)
QOD	Quaque altera die (every other day)
QoL	Quality of Life
QTcF	Corrected QT interval by Fredericia
RA	Rheumatoid Arthritis
RAAS	Renin–angiotensin–aldosterone system
RECIST	Response evaluation criteria in solid tumours
RFS	Relapse-free survival
rhLCAT	Recombinant human Lecithin-cholesterol acyltransferase
RORγ	Related orphan receptor gamma
r/r	Relapsed/refractory
RSV	Respiratory Syncytial Virus
RT	Radiation therapy
SABA	Short-acting beta2-agonist
SAD	Single ascending dose
SAE	Serious adverse event
SBRT	Stereotactic body radiation therapy

List of abbreviations

s.c.	Subcutaneous
SCCHN	Squamous-cell carcinoma of the head and neck
SCLC	Small cell lung cancer
SD	Stable disease
SERD	Selective oestrogen receptor degrader
SGLT2	Sodium-glucose transport protein 2
SGRM	Selective glucocorticoid receptor modulator
SGRQ	Saint George respiratory questionnaire
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
STAT3	Signal transducer and activator of transcription 3
sUA	Serum uric acid
T2DM	Type 2 Diabetes Mellitus
T790M	Threonine 790 substitution with methionine
TACE	Transarterial Chemoembolization
TEAEs	Treatment-emergent adverse events
TESAEs	Treatment-emergent serious adverse events
TID	Ter in die (three times a day)
TJC	Tender joint count
TKI	Tyrosine kinase Inhibitor
TLR	Toll-like receptor 9
TMA	Thrombotic microangiopathy
TNBC	Triple negative breast cancer
TNF	Tumour necrosis factor
TSLP	Thymic stromal lymphopoietin
TTF	Time to treatment failure
TTNT	Time to next therapy
TTP	Time to tumour progression
UACR	Urine albumin creatinine ratio
UMEC	Umeclidinium
URAT1	Uric Acid Transporter 1
UWDRS	Unified Wilson Disease Rating Scale
VEGF	Vascular endothelial growth factor
YTE	Triple-amino-acid (M252Y/S254T/T256E [YTE]) substitution

Clinical trials appendix

Q1 2021 results update