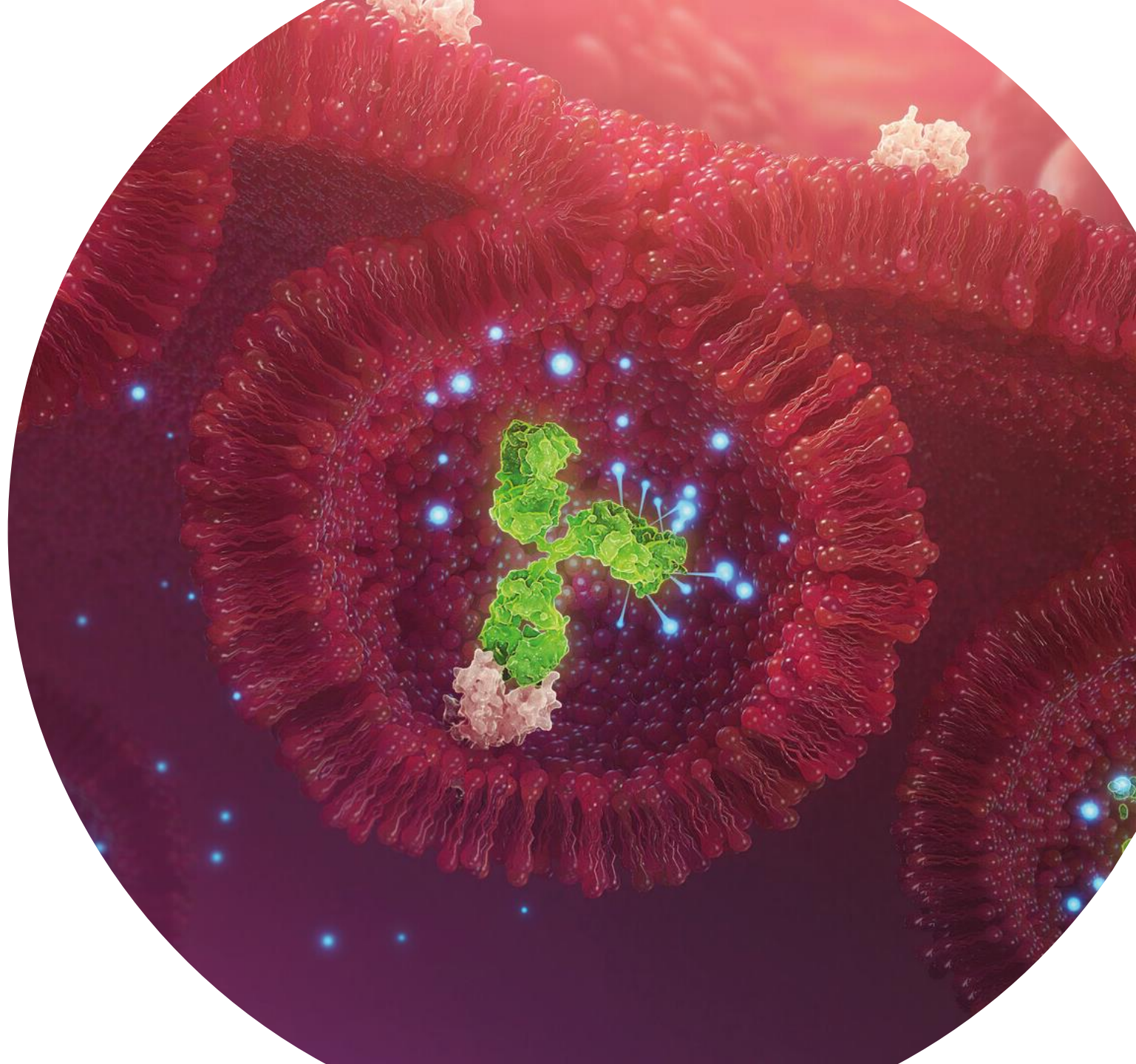




Clinical Trials Appendix

9M and Q3 2023 Results Update



Key upcoming pipeline catalysts: 2023 and 2024

Oncology BioPharmaceuticals Rare Disease

H2 2023

capivasertib – HR+/HER2-neg breast cancer (2L) (CAPitello-291)
eplontersen – hATTR-PN (NEURO-TTRansform) (US)



Regulatory
decision^{1,2}

H1 2024

Tagrisso – EGFRm NSCLC (1L) (FLAURA2)
Imfinzi – biliary tract cancer (TOPAZ-1) (CN)
Imfinzi – NSCLC (neoadjuvant) (AEGEAN)
Enhertu – HER2+ breast cancer (3L) (DESTINY-Breast02) (EU)
Beyfortus – RSV (MELODY-MEDLEY) (JP, CN)
roxadustat – chemotherapy-induced anaemia (CN)
Ultomiris – NMOSD (CHAMPION-NMOSD) (US)
danicopan – PNH with extravascular haemolysis

H2 2024

Enhertu – HER2+ breast cancer (3L) (DESTINY-Breast02) (US)
Fasenra – asthma (MIRACLE) (CN)

Imfinzi – NSCLC (unresectable, Stg. III) ([PACIFIC-2](#))
AZD3152 – prevention of COVID-19 ([SUPERNOVA](#))

Tagrisso – EGFRm NSCLC (unresectable Stg. III) ([LAURA](#))
Imfinzi – SCLC (limited-stage) ([ADRIATIC](#))
Imfinzi – bladder cancer (1L) ([NILE](#))
Enhertu – HER2-low breast cancer (2L) ([DESTINY-Breast06](#))
capivasertib – TNBC (locally adv./met.) ([CAPitello-290](#))
Fasenra – HES ([NATRON](#))
acoramidis – ATTR-CM ([ALXN2060-TAC-302](#))

Tagrisso – EGFRm NSCLC (resectable, Stg. II/III) ([NeoADAURA](#))
Imfinzi – GC/GEJC (resectable) ([MATTERHORN](#))
Imfinzi – liver cancer (adjuvant) ([EMERALD-2](#))
Lynparza – PARP BRCAwt ovarian cancer (1L) ([MONO-OLA1](#))
Orpathys – NSCLC with MET exon 14 mutations (locally adv./met.)
Dato-DXd – TNBC (locally rec. inop./met.) ([TROPION-Breast02](#))
Breztri – moderate asthma ([VATHOS](#))
Breztri – mild to moderate asthma ([LITHOS](#))
Fasenra – CRwNP ([ORCHID](#))
Tezspire – chronic rhinosinusitis with nasal polyps ([WAYPOINT](#))
Tezspire – severe asthma ([DIRECTION](#))
Koselugo – NF1-PN ([KOMET](#))
anselamimab – AL amyloidosis (Mayo Stg. IIIa) ([CAEL101-302](#))



Key Phase III
data readouts

¹Regulatory decision includes programmes under review in a major market

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



Clinical Trials Appendix: selected highlights

BioPharmaceuticals


Saphnelo™
(anifrolumab-fria)
Intravenous Use 300 mg/vial


Fasenra®
(benralizumab) Subcutaneous Injection 30 mg


farxiga (dapagliflozin)


TEZSPIRE™
(tezepelumab-ekko) Subcutaneous Injection 210 mg

tozorakimab (IL-33)

AZD3152 (COVID-19 LAAB)

eplontersen (LICA)

mitiperstat (MPO)

baxdrostat (aldosterone synthase inhibitor)

Oncology


TAGRISSO®
osimertinib


ENHERTU®
fam-trastuzumab deruxtecan-nxki
20 mg/mL INJECTION FOR INTRAVENOUS USE


CALQUENCE®
(acalabrutinib) 100 mg capsules


IMFINZI®
durvalumab
Injection for Intravenous Use 50 mg/mL


Lynparza™
olaparib

Dato-DXd (TROP2 ADC)

capivasertib (AKT)

camizestrant (ngSERD)

saruparib (PARP-1sel)

bispecific mAbs:

volrustomig (PD-1/CTLA-4)

rilvegostomig (PD 1/TIGIT)

sabestomig (PD-1/TIM3)

Rare Disease


ULTOMIRIS®
(ravulizumab-cwvz)

vemircopan (oral Factor D)

gefurulimab (bispecific C5 mini-body)

ALXN1850 (ngHPP)

Approved medicines:
key LCM

Next-wave pipeline



Project movement since Q2 2023 update

New to Phase I	New to Phase II	New to pivotal trial	New to registration
<p><u>NME</u> NT-175 TGFBR2 KO armoured TCR-T targeting TP53 R175H/HLA-A*02:01 solid tumours</p> <p>AZD5863 CLDN18.2 x CD3 bi-specific antibody (HBM7022) solid tumours</p>		<p><u>Additional indication</u> camizestrant CAMBRIA-2 selective estrogen receptor degrader ER+/HER2- early breast cancer</p> <p>volrustomig eVOLVE-Cervical PD-1/CTLA-4 bispecific mAb locally advanced cervical cancer</p> <p>volrustomig eVOLVE-Lung02 PD-1/CTLA-4 bispecific mAb 1L metastatic NSCLC</p> <p>zibotentan/dapagliflozin ZENITH High Proteinuria endothelin A receptor antagonist/SGLT2 inhibitor CKD with high proteinuria</p> <p><u>Life-cycle management</u> Breztri/Trixeo ATHLOS LABA/LAMA/ICS COPD cardiopulmonary exercise trial</p> <p>Saphnelo DAISY# type I IFN receptor mAb systemic sclerosis</p>	<p><u>Life-cycle management</u> Imfinzi + CTx neoadjuvant AEGEAN#1 PD-L1 mAb + CTx locally-advanced (Stage II-III) NSCLC</p> <p>roxadustat#1 hypoxia-inducible factor prolyl hydroxylase inhibitor chemotherapy-induced anaemia</p> <p>Tagrisso + CTx FLAURA2¹ EGFR inhibitor + CTx 1L advanced EGFRm NSCLC</p>

Phase progressions based on first subject in achievement

#Partnered and/or in collaboration ¹Submission accepted ²Approved ³Completed

4 As of 9 November 2023.

Appendix: [Glossary](#).



Project movement since Q2 2023 update

Removed from Phase I	Removed from Phase II	Removed from Phase III	Removed from registration
	<p><u>NME</u> tarperprumig anti-properdin bi-specific SCD</p> <p>vemircopan oral factor D inhibitor PNH</p> <p><u>Additional indication</u> ceralasertib + Imfinzi MONETTE¹ ATR inhibitor + PDL-1 melanoma</p> <p>oleclumab+CTx or Imfinzi+oleclumab+CTx³ CD73 mAb + CTx or PD-L1 mAb + CD73 mAb + CTx metastatic pancreatic cancer</p> <p><u>Life-cycle management</u> Imfinzi (platform) COAST^{#3} PD-L1 mAb + multiple novel oncology therapies NSCLC</p> <p>Imfinzi (platform) NeoCOAST^{#3} PD-L1 mAb + multiple novel oncology therapies NSCLC</p> <p>Imfinzi + Lynparza ORION^{#3} PD-L1 mAb + PARP inhibitor 1L metastatic NSCLC</p>		<p><u>Life-cycle management</u> Enhertu DESTINY-Breast02^{#2} HER2-targeting ADC HER2+ unresectable and/or metastatic breast cancer pre-treated with prior standard of care HER2 therapies, including T-DM1</p>

Phase progressions based on first subject in achievement

[#]Partnered and/or in collaboration ¹Submission accepted ²Approved ³Completed

5 As of 9 November 2023.

Appendix: [Glossary](#).



Q3 2023 Oncology new molecular entity¹ pipeline

Phase I 9 New Molecular Entities	Phase II 11 New Molecular Entities	Phase III 19 New Molecular Entities	
AZD0486 CD19-CD3 TCE R/R B-Cell non-Hodgkin lymphoma	AZD0171 + <i>Imfinzi</i> + CTx anti-LIF+PD-L1+CTx 1L metastatic PDAC	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
AZD1390 ATM glioblastoma	AZD8205 B7-H4 targeting ADC solid tumours	camizestrant CAMBRIA-1 SERD HR+ HER2- extended adjuvant breast cancer	camizestrant CAMBRIA-2 selective estrogen receptor degrader ER+/HER2- early breast cancer
AZD5335 anti-folate receptor alpha topoisomerase 1 inhibitor ADC ovarian cancer, lung adenocarcinoma	camizestrant SERD HR+ breast cancer	capiasertib + abiraterone CAPItello-281 AKT+abiraterone PTEN deficient mHSPC	capiasertib + CTx CAPItello-290 AKT+chemotherapy 1L mTNBC
AZD5863 CLDN18.2 x CD3 bi-specific antibody (HBM7022) solid tumours	capiasertib AKT prostate cancer	capiasertib + docetaxel CAPItello-280 AKT+Docetaxel mCRPC prostate cancer	capiasertib + <i>Faslodex</i> + palbociclib CAPItello-292 AKT+fulvestrant+CDK4/6 1L triplet in early relapse/ET resistant locally advanced or mBC
AZD9574 PARP inhibitor advanced solid malignancies	ceralasertib ATR solid tumours	ceralasertib + <i>Imfinzi</i> LATIFY ATR inhibitor + PDL-1 non-small cell lung cancer	datopotamab deruxtecan TROPION-Breast02# TROP2 ADC 1L TNBC
AZD9592 EGFR/cMET solid tumours	<i>Imfinzi</i> + monalizumab# PD-L1+NKG2A solid tumours	datopotamab deruxtecan TROPION-Breast01# TROP2 ADC 2-3L HR+ HER2- breast cancer	datopotamab deruxtecan TROPION-Lung01# TROP2 ADC 2L+ NSCLC with or without actionable genomic alterations
NT-125 autologous, fully-individualized, multi-specific TCR therapy targeting neoantigens solid tumours	IPH5201 + <i>Imfinzi</i> # CD39 + PD-L1 neo-adjuvant/adjuvant NSCLC	datopotamab deruxtecan TROPION-Breast03# TROP2 ADC adjuvant residual disease TNBC	datopotamab deruxtecan TROPION-Lung08# TROP2 ADC 1L metastatic NSCLC
NT-175 TGFBR2 KO armoured TCR-T targeting TP53 R175H/HLA-A*02:01 solid tumours	rilvegostomig ARTEMIDE-01# PD1/TIGIT bispecific mAb solid tumours	datopotamab deruxtecan TROPION-Lung07# TROP 2 ADC 1L NSCLC PD-L1 <50% non-squamous	<i>Imfinzi</i> +/- oleclumab +/- monalizumab PACIFIC-9# PD-L1+NKG2A or PD-L1+CD73 unresectable stage III NSCLC
volrustomig + lenvatinib PD-1/CTLA-4+VEGF advanced RCC	sabestomig (AZD7789) PD1/TIM3 bispecific mAb solid tumours, haematological malignancies	datopotamab deruxtecan AVANZAR# TROP 2 ADC 1L NSCLC, squamous and non-squamous 1L NSCLC, TROP2 BM+	volrustomig eVOLVE-Cervical PD-1/CTLA-4 bispecific mAb locally advanced cervical cancer
	saruparib (AZD5305) PARP1Sel solid tumours	volrustomig eVOLVE-Meso PD-1/CTLA-4 bispecific mAb unresectable malignant pleural mesothelioma	
	volrustomig PD-1/CTLA-4 solid tumours		
			Under review 1 New Molecular Entity
			capiasertib + <i>Faslodex</i> CAPItello-291 AKT+fulvestrant 2L+ in AI-resistant locally advanced or mBC

Phase progressions based on first subject in achievement

¹Includes additional indications for assets where the lead is not yet launched

#Partnered and/or in collaboration *Registrational Phase II trial

6 As of 9 November 2023.

Appendix: [Glossary](#).

● Precision medicine approach being explored



Q3 2023 Oncology lifecycle management¹ pipeline

Phase I 1 Project	Phase II 8 Projects	Phase III 31 Projects		
<i>Enhertu</i> (platform) DESTINY-Breast08# HER2 ADC HER2-low breast cancer	<i>Enhertu</i> (platform) DESTINY-Breast07# HER2 ADC HER2+ breast cancer	<i>Calquence</i> + R-CHOP ESCALADE BTK+R-CHOP 1L DLBCL	<i>Calquence</i> + venetoclax + obinutuzumab AMPLIFY# BTK+BCL-2+anti-CD20 1L CLL	<i>Calquence</i> ECHO# BTK inhibitor 1L MCL
	<i>Enhertu</i> DESTINY-PanTumour01# HER2 ADC HER2 mutant tumours	<i>Enhertu</i> DESTINY-Breast05# HER2 ADC HER2+ post-neoadjuvant high-risk breast cancer	<i>Enhertu</i> DESTINY-Breast11# HER2 ADC neoadjuvant HER2+ breast cancer	<i>Enhertu</i> DESTINY-Breast06# HER2 ADC post-ET HER2-low/HR+ breast cancer 2L
	<i>Enhertu</i> DESTINY-PanTumour02# HER2 ADC HER2 expressing solid tumours	<i>Enhertu</i> DESTINY-Breast09# HER2 ADC HER2+ breast cancer 1L	<i>Enhertu</i> DESTINY-Gastric04# HER2 ADC HER2+ gastric 2L	<i>Enhertu</i> DESTINY-Lung04# HER2 ADC HER2m NSCLC 1L
	<i>Imfinzi</i> (platform) BEGONIA PD-L1 1L metastatic TNBC	<i>Imfinzi</i> + CRT PACIFIC-5 (China)# PD-L1+CRT locally-advanced stage III NSCLC	<i>Imfinzi</i> + CRT KUNLUN# PD-L1+CRT locally-advanced ESCC	<i>Imfinzi</i> + CRT PACIFIC-2# PD-L1+CRT locally-advanced stage III NSCLC
	<i>Imfinzi</i> (platform) HUDSON PD-L1+multiple novel ONC therapies post IO non-small cell lung cancer	<i>Imfinzi</i> + CTx NIAGARA PD-L1+CTx muscle invasive bladder cancer	<i>Imfinzi</i> + domvanalimab (AB154) PACIFIC-8# PD-L1+TIGIT+CTx unresectable stage III NSCLC	<i>Imfinzi</i> + EV +/- <i>Imjudo</i> VOLGA PD-L1 + nectin-4 targeting ADC +/- CTLA4 MIBC
	<i>Lynparza</i> (basket) LYNK002# PARP HRRm cancer	<i>Imfinzi</i> + <i>Imjudo</i> + SoC NILE PD-L1+CTLA-4+SoC 1L urothelial cancer	<i>Imfinzi</i> + FLOT MATTERHORN# PD-L1+CTx neoadjuvant/adjutant gastric cancer	<i>Imfinzi</i> + <i>Imjudo</i> + TACE +/- lenvatinib EMERALD-3 PD-L1+CTLA4+VEGF+/-chemo-embolization locoregional HCC
	<i>Tagrisso</i> + <i>Orpathys</i> SAVANNAH# EGFR+MET advanced EGFRm NSCLC	<i>Imfinzi</i> + VEGF + TACE EMERALD-1# PD-L1+VEGF+TACE locoregional HCC	<i>Imfinzi</i> + VEGF EMERALD-2# PD-L1+VEGF adjuvant HCC	<i>Imfinzi</i> +/- <i>Imjudo</i> + CRT ADRIATIC# PD-L1+/-CTLA-4+CRT 1L LS-SCLC
	<i>Tagrisso</i> ORCHARD platform study# EGFR+multiple novel ONC therapies 2L EGFRm osimertinib-resistant NSCLC	<i>Imfinzi</i> POTOMAC PD-L1 non-muscle invasive bladder cancer	<i>Imfinzi</i> post-SBRT PACIFIC-4# PD-L1 mAb post-SBRT stage I/II NSCLC	<i>Lynparza</i> + <i>Imfinzi</i> + bevacizumab DUO-O# PARP+PD-L1+VEGF 1L ovarian cancer
		<i>Lynparza</i> + <i>Imfinzi</i> DUO-E# PARP+PD-L1 1L endometrial cancer	<i>Lynparza</i> MONO-OLA1# PARP 1L BRCAwt ovarian cancer	<i>Orpathys</i> + <i>Imfinzi</i> SAMETA# MET+PD-L1 1L papillary renal cell carcinoma
		<i>Tagrisso</i> + <i>Orpathys</i> SAFFRON# EGFR + MET advanced EGFRm non-small cell lung cancer	<i>Tagrisso</i> LAURA EGFR inhibitor stage III EGFRm NSCLC	<i>Tagrisso</i> +/- CTx neoadjuvant NeoADAURA EGFR+/-CTx stage II/III resectable EGFRm NSCLC
		<i>Tagrisso</i> ADAURA2 EGFR adjuvant EGFRm NSCLC stage Ia2-Ia3 following complete tumour resection		
			Under review 2 Projects	
			<i>Imfinzi</i> + CTx neoadjuvant AEGEAN PD-L1+CTx locally-advanced stage II-III NSCLC	<i>Tagrisso</i> + CTx FLAURA2 EGFR+chemo 1L adv EGFRm NSCLC

Phase progressions based on first subject in achievement

¹Includes significant lifecycle management projects and parallel indications for assets beyond Phase III

#Partnered and/or in collaboration [†]Registrational Phase I/III trial

7 As of 9 November 2023.

Appendix: [Glossary](#).

● Precision medicine approach being explored



Q3 2023 BioPharmaceuticals new molecular entity¹ pipeline

Phase I 14 New Molecular Entities	Phase II 13 New Molecular Entities	Phase III 5 New Molecular Entities	Under review 1 New Molecular Entity
AZD0780 PCSK9 dyslipidemia	atuliflapon FLAP asthma	AZD3152 SUPERNOVA ¹ SARS-CoV-2 LAAB prevention of COVID-19	eplontersen# LICA hATTR-polyneuropathy
AZD2373 podocyte health nephropathy	AZD2693 NASH resolution non-alcoholic steatohepatitis	eplontersen# LICA ATTR-cardiomyopathy	
AZD4041# orexin 1 receptor antagonist opioid use disorder	AZD3427 relaxin mimetic heart failure	tozorakimab OBERON TITANIA PROSPERO MIRANDA IL-33 COPD	
AZD4604 inhaled JAK1 asthma	balcinrenone/dapagliflozin MR+SGLT2 heart failure with CKD	tozorakimab TILIA IL-33 severe viral lower respiratory tract disease	
AZD5055 porcupine inhibitor idiopathic pulmonary fibrosis	baxdrostat aldosterone synthase inhibitor hypertension	zibotentan/dapagliflozin endothelin A receptor antagonist/SGLT2 high proteinuria CKD	
AZD5462# RXFP1 agonist heart failure	MEDI1341# alpha synuclein mAb multiple system atrophy/Parkinson's disease		
AZD6234 peptide obesity with related comorbidities	MEDI6570 LOX-1 CV disease		
AZD6793 IRAK4 inhibitor inflammatory diseases	MEDI7352 NGF/TNF OA pain / PDN		
AZD7503 ASO non-alcoholic steatohepatitis	mitiperstat MPO HFpEF / NASH		
AZD7798 humanised monoclonal antibody targets T cells subset Crohn's disease	mitiperstat myeloperoxidase COPD		
AZD8630# inhaled TSLP FAb asthma	tozorakimab IL-33 diabetic kidney disease		
AZD9550 GLP-1R glucagon dual agonist non-alcoholic steatohepatitis	tozorakimab FRONTIER 3 IL-33 asthma		
MEDI0618* PAR2 antagonist migraine	zibotentan/dapagliflozin endothelin A receptor antagonist/SGLT2 CKD and liver cirrhosis		
MEDI1814# amyloid beta mAb Alzheimer's disease			

Phase progressions based on first subject in achievement ¹Includes additional indications for assets where the lead is not yet launched

#Partnered and/or in collaboration *Phase I/IIa ¹Registrational Phase I/III trial

8 As of 9 November 2023.

Appendix: [Glossary](#).

● Precision medicine approach being explored



Q3 2023 BioPharmaceuticals lifecycle management¹ pipeline

Phase I 0 Projects	Phase II 2 Projects	Phase III 14 Projects	Under review 1 Project
	<p><i>Andexxa</i> anti-factor Xa reversal urgent surgery</p>	<p><i>Breztri/Triexo</i> (PT010) KALOS LOGOS LABA/LAMA/ICS asthma</p>	<p>roxadustat # HIFPH anaemia chemotherapy induced anaemia</p>
	<p><i>Tezspire</i> COURSE# TSLP chronic obstructive pulmonary disease</p>	<p><i>Breztri/Triexo</i> ATHLOS LABA/LAMA/ICS COPD cardiopulmonary exercise trial</p>	
		<p><i>Farxiga/Forxiga</i> DAPA-MI SGLT-2 prevention of HF and CV death following a myocardial infarction</p>	
		<p><i>Fasenra</i> RESOLUTE# IL-5R chronic obstructive pulmonary disease</p>	
		<p><i>Fasenra</i> MANDARA IL-5R eosinophilic granulomatosis with polyangiitis</p>	
		<p><i>Fasenra</i> NATRON IL-5R hypereosinophilic syndrome</p>	
		<p><i>Fasenra</i> ORCHID# IL-5R nasal polyps</p>	
		<p><i>Lokelma</i> DIALIZE-Outcomes potassium binder CV outcomes in patients on chronic haemodialysis with hyperkalaemia</p>	
		<p><i>Lokelma</i> STABILIZE-CKD potassium binder hyperkalaemia in CKD</p>	
		<p><i>Saphnelo</i> DAISY# Type I IFN receptor mAb systemic sclerosis</p>	
		<p><i>Saphnelo</i> IRIS# Type I IFN receptor mAb lupus nephritis</p>	
		<p><i>Saphnelo</i> TULIP-SC# Type I IFN receptor SLE SC</p>	
		<p><i>Tezspire</i> WAYPOINT# TSLP nasal polyps</p>	
		<p><i>Tezspire</i> CROSSING# TSLP eosinophilic esophagitis</p>	

Phase progressions based on first subject in achievement

¹Includes significant lifecycle management projects and parallel indications for assets beyond Phase III

#Partnered and/or in collaboration ⁴Registrational Phase I/III trial

9 As of 9 November 2023.

Appendix: [Glossary](#).

● Precision medicine approach being explored



Q3 2023 Rare Disease pipeline¹

Phase I 6 Projects	Phase II 5 Projects	Phase III 5 Projects	Under review 1 Projects
ALXN1850 next gen TNSALP ERT hypophosphatasia	danicopan factor D geographic atrophy	acoramidis# oral TTR stabilizer transthyretin amyloid cardiomyopathy	danicopan factor D PNH with clinically significant extravascular haemolysis
ALXN1910 next gen TNSALP ERT bone metabolism	vemircopan oral factor D inhibitor generalized myasthenia gravis	anselamimab (CAEL-101) fibril-reactive mAb AL amyloidosis	
ALXN1920 kidney-targeted factor H fusion protein nephrology	vemircopan oral factor D proliferative lupus nephritis or immunoglobulin A nephropathy	gefurulimab humanised bispecific VHH antibody generalised myasthenia gravis	
ALXN2030 siRNA targeting complement C3 nephrology	<i>Ultomiris</i> [‡] anti-complement C5 mAb dermatomyositis	<i>Ultomiris</i> anti-complement C5 mAb haematopoietic stem cell transplant-associated thrombotic microangiopathy	
ALXN2080 oral factor D healthy volunteers	<i>Ultomiris</i> anti-complement C5 mAb proliferative lupus nephritis or immunoglobulin A nephropathy	<i>Ultomiris</i> ARTEMIS anti-complement C5 mAb cardiac surgery-associated acute kidney injury	
ALXN2220 (NI006)# TTR depleter transthyretin amyloid cardiomyopathy			

Phase progressions based on first subject in achievement

¹Includes new molecular entities and significant lifecycle management projects

#Partnered and/or in collaboration [‡]Registrational Phase II/III trial

As of 9 November 2023.

Appendix: [Glossary](#).

● Precision medicine approach being explored



Designations in our pipeline

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Accelerated approvals

Andexxa Acute Major Bleed (US)
Calquence MCL 1L (US)
Beyfortus RSV mAb-YTE (EU)

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Breakthrough / PRIME¹ / Sakigake²

Beyfortus RSV mAb-YTE MELODY-MEDLEY (US)
Beyfortus RSV mAb-YTE MELODY-MEDLEY (CN)
Beyfortus RSV mAb-YTE MELODY-MEDLEY (EU) ¹
Calquence MCL 1L (US)
danicopan PNH-EVH (US)
danicopan PNH-EVH (EU)
Enhertu HER2 overexp tumors DESTINY-PanTumor02 (US)
Enhertu HER2+/HER2-low gastric 3L DESTINY-Gastric01 (US)
Enhertu HER2+/HER2-low gastric 3L DESTINY-Gastric01 (JP) ²
Enhertu HER2mut NSCLC 2L+ DESTINY-Lung01 (US)
Tagrisso + CTx EGFRm NSCLC 1L FLAURA2 (US)
Tezspire asthma NAVIGATOR (US)

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Fast Track

anselamibab AL amyloidosis (US)
AZD3427 relaxin mimetic heart failure (US)
Beyfortus RSV mAb-YTE MELODY-MEDLEY (US)
camizestrant 1L HR+ HER2- ESR1m breast cancer SERENA-6 (US)
capivasertib+fulv HR+ breast 2L+ CAPItello-291 (US)
Lokelma ESRD DIALIZE-OUTCOMES (US)
Orpathys + Tagrisso NSCLC SAVANNAH/SAFFRON (US)
Saphnelo SLE (US)
tozorakimab severe viral lower respiratory tract disease (US)

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Priority Review

Beyfortus RSV mAb-YTE MELODY-MEDLEY (CN)
Calquence MCL 1L (US)
capivasertib+fulv HR+ breast 2L+ CAPItello-291 (US)
Enhertu HER2+/HER2-low gastric 3L DESTINY-Gastric01 (US)
Enhertu HER2mut NSCLC 2L+ DESTINY-Lung01 (US)
Imfinzi + CTx BTC 1L (TOPAZ-1) (US)
Imfinzi + Imjudo HCC 1L (HIMALAYA) (US)
Lynparza + abiraterone all-comers mCRPC 1L PROpel (US)
Lynparza gBRCA adj breast OlympiA (US)
Roxadustat chronic kidney disease (CN)
Tagrisso + CTx EGFRm NSCLC 1L FLAURA2 (US)
Tezspire asthma NAVIGATOR (US)
Ultomiris gMG (US)

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Orphan

ALXN2220 ATTR-CM (US)
Andexxa Acute Major Bleed (JP)
anselamibab AL amyloidosis (US)
anselamibab AL amyloidosis (EU)
Calquence CLL 1L (US)
Calquence CLL 1L (EU)
Calquence MCL 1L (US)
danicopan PNH (US)
danicopan PNH (EU)
Enhertu HER2+/HER2-low gastric 3L DESTINY-Gastric01 (US)
eplontersen transthyretin-mediated amyloidosis (EU)
eplontersen transthyretin-mediated amyloidosis (US)
Fasenra EGPA MANDARA (US)
Fasenra HES NATRON (US)
gefurulimab myasthenia gravis (US)
Imfinzi + CTx Biliary Tract 1L TOPAZ-1 (US)
Imfinzi + CTx Biliary Tract 1L TOPAZ-1 (JP)
Imfinzi +/- Imjudo HCC 1L HIMALAYA (EU)
Imfinzi +/- Imjudo HCC 1L HIMALAYA (US)
Lynparza gBRCA adj breast OlympiA (JP)
Tezspire EoE CROSSING (US)
Ultomiris DM (US)
Ultomiris HSCT-TMA (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. ¹PRIME is a scheme launched by the EMA to enhance support for the development of medicines that target an unmet medical need. ²SAKIGAKE is aimed at early introduction of innovative medicines, medical devices, etc. that are initially developed in Japan

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

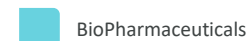
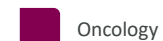
PRIORITY REVIEW DESIGNATION is the US FDA's goal to take action on an application within 6 months

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

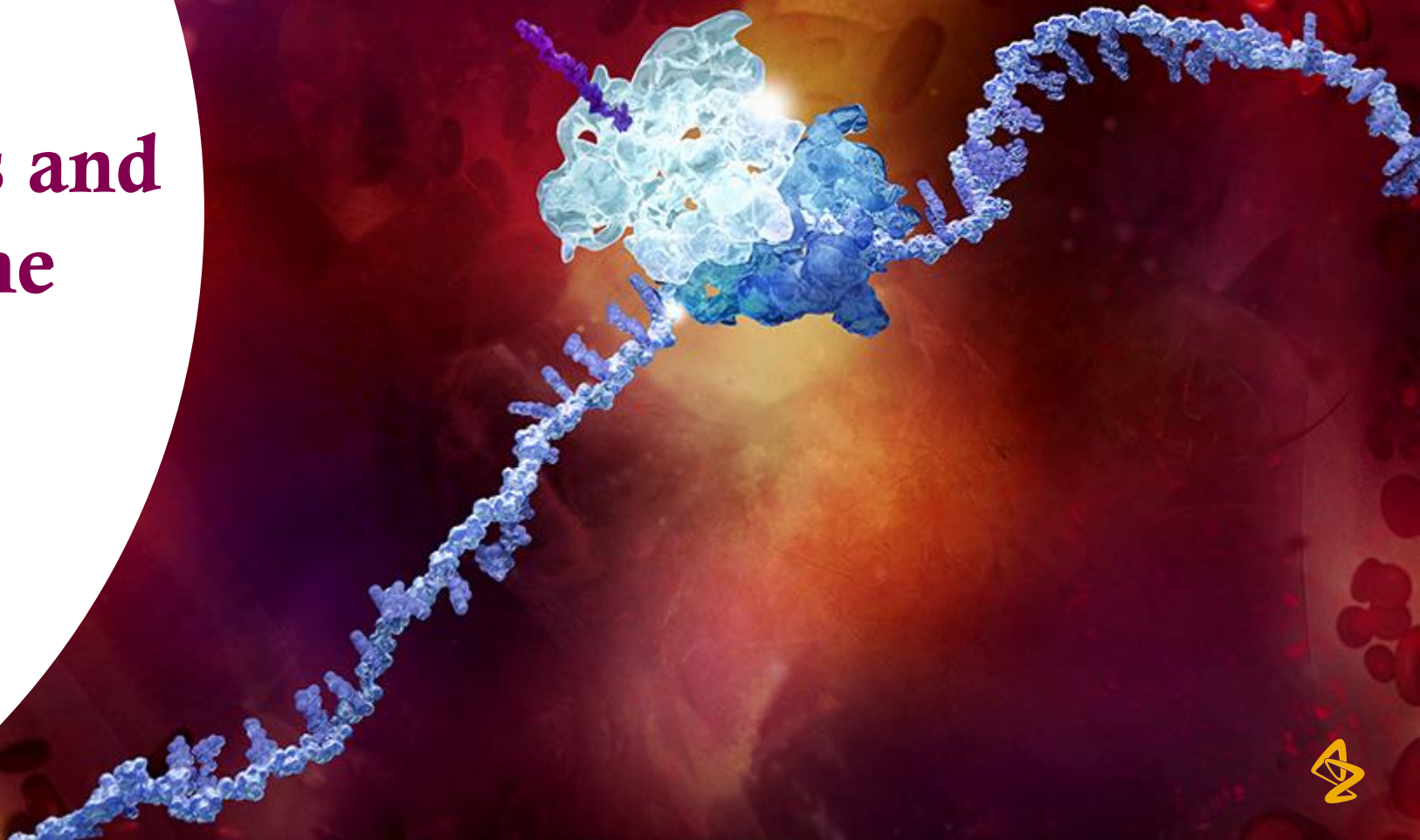
NOTE: excludes designations for projects which have launched in all applicable major markets

11 As of 9 November 2023.

Appendix: [Glossary](#).



**Oncology:
approved medicines and
late-stage pipeline**



Tagrisso (highly-selective, irreversible EGFRi)

NSCLC

Approved medicines

Late-stage development

Early development

Oncology

CVRM

R&I

Other

V&I

Rare Disease

Trial	Population	Patients	Design	Endpoints	Status
Phase III LAURA NCT03521154	Maintenance therapy in patients with locally advanced, unresectable EGFRm Stage III NSCLC whose disease has not progressed following platinum-based chemoradiation therapy	200	<ul style="list-style-type: none"> Arm 1: <i>Tagrisso</i> Arm 2: placebo Global trial – 17 countries 	<ul style="list-style-type: none"> Primary endpoint: PFS (BICR) Secondary endpoints: CNS PFS, OS, DoR, ORR and DCR 	<ul style="list-style-type: none"> FPCD: Q4 2018 LPCD: Q3 2022 Data anticipated: H1 2024
Phase III ADAURA2 NCT05120349	Adjuvant EGFRm NSCLC Stage IA2 to IA3 following complete tumour resection	380	<ul style="list-style-type: none"> Arm 1: <i>Tagrisso</i> Arm 2: placebo 	<ul style="list-style-type: none"> Primary endpoint: DFS Secondary endpoints: DFS Rate, OS, OS rate and QoL 	<ul style="list-style-type: none"> FPCD: Q2 2022 Data anticipated: >2024



Tagrisso (highly-selective, irreversible EGFRi)

NSCLC, combinations

Trial	Population	Patients	Design	Endpoints	Status
Phase III NeoADAURA NCT04351555	Neoadjuvant EGFRm NSCLC	351	<ul style="list-style-type: none"> Arm 1: placebo + pemetrexed/carboplatin or pemetrexed/cisplatin Arm 2: <i>Tagrisso</i> + pemetrexed/carboplatin or pemetrexed/cisplatin Arm 3: <i>Tagrisso</i> Global trial – 23 countries 	<ul style="list-style-type: none"> Primary endpoint: mPR Secondary endpoints: cPR, EFS, DFS and OS 	<ul style="list-style-type: none"> FPCD: Q1 2021 Data anticipated: H2 2024
Phase III FLAURA2 NCT04035486	1L EGFRm NSCLC	586	<ul style="list-style-type: none"> Arm 1: <i>Tagrisso</i> + pemetrexed/carboplatin or pemetrexed/cisplatin Arm 2: <i>Tagrisso</i> Global trial – 23 countries 	<ul style="list-style-type: none"> Primary endpoint: PFS Secondary endpoints: OS, LOS, ORR DoR, depth of response, PFS2, QoL and PK parameters 	<ul style="list-style-type: none"> FPCD: Q4 2019 Data readout: Q2 2023 Primary endpoint met
Phase III COMPEL NCT04765059	EGFRm metastatic NSCLC patients who have progressed extracranially following 1L treatment with <i>Tagrisso</i>	204	<ul style="list-style-type: none"> Arm 1: <i>Tagrisso</i> + pemetrexed/carboplatin or pemetrexed/cisplatin Arm 2: placebo + pemetrexed/carboplatin or pemetrexed/cisplatin Global trial 	<ul style="list-style-type: none"> Primary endpoint: PFS Secondary endpoints: intracranial PFS, extracranial PFS and OS 	<ul style="list-style-type: none"> FPCD: Q3 2021 Data anticipated: H2 2024
Phase III SAFFRON NCT05261399 Partnered (HUTCHMED)	EGFR-mutated, MET-overexpressed and/or amplified, locally advanced or metastatic NSCLC patients who have progressed on first- or second-line treatment with <i>Tagrisso</i>	324	<ul style="list-style-type: none"> Arm 1: <i>Tagrisso</i> + <i>Orpathys</i> Arm 2: pemetrexed with either cisplatin or carboplatin 	<ul style="list-style-type: none"> Primary endpoint: PFS Secondary endpoints: OS, ORR, PK, DCR and DoR 	<ul style="list-style-type: none"> FPCD: Q3 2022 Data anticipated: >2024
Phase III SANOVO NCT05009836 Partnered (HUTCHMED)	1L EGFRm, MET+ locally advanced or metastatic NSCLC	320	<ul style="list-style-type: none"> Arm 1: <i>Tagrisso</i> + <i>Orpathys</i> Arm 2: <i>Tagrisso</i> + placebo 	<ul style="list-style-type: none"> Primary endpoint: PFS 	<ul style="list-style-type: none"> FPCD: Q3 2021 Data anticipated: H2 2024



Tagrisso (highly-selective, irreversible EGFRi)

NSCLC, combinations

Trial	Population	Patients	Design	Endpoints	Status
Phase III SACHI NCT05015608 Partnered (HUTCHMED)	Locally advanced or metastatic NSCLC with MET amplification after failure of the first-line EGFR inhibitor therapy	250	<ul style="list-style-type: none"> Arm 1: <i>Tagrisso</i> + <i>Orpathys</i> Arm 2: pemetrexed + platinum 	<ul style="list-style-type: none"> Primary endpoint: PFS 	<ul style="list-style-type: none"> FPCD: Q3 2021 Data anticipated: H2 2024
Phase II SAVANNAH NCT03778229	EGFRm/MET+, locally advanced or metastatic NSCLC who have progressed following treatment with <i>Tagrisso</i>	360	<ul style="list-style-type: none"> Protocol v1-6: single-arm, open-label trial Protocol v7: randomised, double-blind trial Arm 1: <i>Tagrisso</i> + <i>Orpathys</i> Arm 2: placebo + <i>Orpathys</i> Global trial 	<ul style="list-style-type: none"> Primary endpoint: ORR Secondary endpoints: PFS, DoR and OS 	<ul style="list-style-type: none"> FPCD: Q1 2019 Data anticipated: H1 2024 Initial data readout: Q2 2020
Phase II ORCHARD NCT03944772	Advanced EGFRm NSCLC patients who have progressed on first-line <i>Tagrisso</i> treatment	250	<ul style="list-style-type: none"> Modular design platform trial: Module 1: <i>Tagrisso</i> + <i>Orpathys</i> (cMET) Module 2: <i>Tagrisso</i> + gefitinib (EGFRm) Module 3: <i>Tagrisso</i> + necitumumab (EGFRm) Module 4: carboplatin + pemetrexed + <i>Imfinzi</i> Module 5: <i>Tagrisso</i> + alectinib (ALK) Module 6: <i>Tagrisso</i> + selpercatinib (RET fusion) Module 7: <i>Imfinzi</i> + etoposide + carboplatin or cisplatin Module 8: <i>Tagrisso</i> + pemetrexed + carboplatin or cisplatin Module 9: <i>Tagrisso</i> + <i>Koselugo</i> Module 10: <i>Tagrisso</i> + datopotamab deruxtecan No intervention: observational cohort Global trial – 9 countries 	<ul style="list-style-type: none"> Primary endpoint: ORR Secondary endpoints: PFS, DoR, OS, safety and tolerability 	<ul style="list-style-type: none"> FPCD: Q3 2019 Data anticipated: >2024



Imfinzi (PD-L1 mAb)

Gastrointestinal cancer

Trial	Population	Patients	Design	Endpoints	Status
Phase III EMERALD-1 NCT03778957	Locoregional HCC	710	<ul style="list-style-type: none"> Arm 1: TACE in combination with <i>Imfinzi</i> Arm 2: TACE in combination with <i>Imfinzi</i> + bevacizumab Arm 3: TACE in combination with placebo 	<ul style="list-style-type: none"> Primary endpoint: PFS (Arm 2 vs. Arm 3) Secondary endpoints: PFS (Arm 1 vs. Arm 3) and OS 	<ul style="list-style-type: none"> FPCD: Q1 2019 LPCD: Q3 2021 Data readout: Q4 2023 Primary endpoint met
Phase III EMERALD-2 NCT03847428	HCC (adjuvant)	908	<ul style="list-style-type: none"> Arm 1: <i>Imfinzi</i> + bevacizumab Arm 2: <i>Imfinzi</i> + placebo Arm 3: placebo + placebo 	<ul style="list-style-type: none"> Primary endpoint: RFS (Arm 1 vs. Arm 3) Secondary endpoints: RFS (Arm 2 vs. Arm 3), OS and RFS at 24 months 	<ul style="list-style-type: none"> FPCD: Q2 2019 LPCD: Q2 2022 Data anticipated: H2 2024
Phase III KUNLUN NCT04550260	Locally advanced, unresectable ESCC	640	<ul style="list-style-type: none"> Arm 1: <i>Imfinzi</i> + definitive CRT Arm 2: placebo + definitive CRT 	<ul style="list-style-type: none"> Primary endpoint: PFS Secondary endpoint: OS 	<ul style="list-style-type: none"> FPCD: Q4 2020 LPCD: Q3 2023 Data anticipated: >2024
Phase III MATTERHORN NCT04592913	Resectable GC/GEJC	900	<ul style="list-style-type: none"> Arm 1: <i>Imfinzi</i> + FLOT Arm 2: placebo + FLOT 	<ul style="list-style-type: none"> Primary endpoint: EFS Secondary endpoints: OS (Arm 1 vs. Arm 2) and pCR (Arm 1 vs. Arm 2) 	<ul style="list-style-type: none"> FPCD: Q4 2020 LPCD: Q3 2022 Data anticipated: H2 2024
Phase III HIMALAYA NCT03298451	1L HCC	1324	<ul style="list-style-type: none"> Arm 1: <i>Imfinzi</i> + <i>Imjudo</i> Arm 2: <i>Imfinzi</i> Arm 3: sorafenib 	<ul style="list-style-type: none"> Primary endpoint: OS Secondary endpoints: PFS, TTP and ORR 	<ul style="list-style-type: none"> FPCD: Q4 2017 LPCD: Q4 2019 Data readout: Q4 2021
Phase III TOPAZ-1 NCT03875235	1L BTC	810	<ul style="list-style-type: none"> Arm 1: <i>Imfinzi</i> + gemcitabine + cisplatin Arm 2: placebo + gemcitabine + cisplatin Global trial 	<ul style="list-style-type: none"> Primary endpoint: OS Secondary endpoints: PFS, ORR and DoR 	<ul style="list-style-type: none"> FPCD: Q2 2019 LPCD: Q4 2020 Data readout: Q4 2021
Phase III EMERALD-3 NCT05301842	Locoregional HCC	525	<ul style="list-style-type: none"> Arm 1: TACE + T300 + D + lenvatinib Arm 2: TACE + T300 + D Arm 3: TACE 	<ul style="list-style-type: none"> Primary endpoint: PFS Secondary endpoint: OS 	<ul style="list-style-type: none"> FPCD: Q2 2022 Data anticipated: >2024



Imfinzi (PD-L1 mAb)

Lung cancer

Trial	Population	Patients	Design	Endpoints	Status
Phase III AEGEAN NCT03800134	Neoadjuvant NSCLC patients, Stage II and III resected NSCLC (incl. EGFR/ALK-positive)	800	<ul style="list-style-type: none"> Arm 1: <i>Imfinzi</i> + platinum-based chemotherapy Arm 2: placebo + platinum-based chemotherapy 	<ul style="list-style-type: none"> Primary endpoints: pCR and EFS Secondary endpoints: mPR and DFS 	<ul style="list-style-type: none"> FPCD: Q1 2019 Data readout: Q1 2023
Phase III ADJUVANT BR.31 NCT02273375 Partnered (CCTG)	Adjuvant NSCLC patients, Stage Ib (≥ 4 cm) – Stage IIIa resected (incl. EGFR/ALK-positive)	1360	<ul style="list-style-type: none"> Arm 1: <i>Imfinzi</i> mg/kg i.v. Q4W x 12 months Arm 2: placebo Global trial 	<ul style="list-style-type: none"> Primary endpoint: DFS Secondary endpoint: OS 	<ul style="list-style-type: none"> FPCD: Q1 2015 LPCD: Q1 2020 Data anticipated: H1 2024
Phase III PACIFIC-2 NCT03519971	Unresected, locally advanced NSCLC	300	<ul style="list-style-type: none"> Arm 1: <i>Imfinzi</i> i.v. Q4W + chemotherapy/RT Arm 2: placebo + chemotherapy/RT Global trial (ex-US) 	<ul style="list-style-type: none"> Primary endpoint: PFS Secondary endpoints: OS and ORR 	<ul style="list-style-type: none"> FPCD: Q2 2018 LPCD: Q3 2019 Data anticipated: H2 2023
Phase III PACIFIC-4 NCT03833154	<i>Imfinzi</i> with SBRT in unresected, Stage I/II NSCLC	630	<ul style="list-style-type: none"> Arm 1: <i>Imfinzi</i> i.v. Q4W with definitive SBRT Arm 2: placebo with definitive SBRT 	<ul style="list-style-type: none"> Primary endpoint: PFS Secondary endpoint: OS 	<ul style="list-style-type: none"> FPCD: Q2 2019 Data anticipated: >2024
Phase III PACIFIC-5 NCT03706690	Unresected, locally advanced NSCLC	360	<ul style="list-style-type: none"> Arm 1: <i>Imfinzi</i> i.v. Q4W following chemotherapy/RT Arm 2: placebo following chemotherapy/RT Global trial (ex-US with China focus) 	<ul style="list-style-type: none"> Primary endpoint: PFS Secondary endpoint: OS 	<ul style="list-style-type: none"> FPCD: Q1 2019 Data anticipated: H2 2024
Phase III PACIFIC-8 NCT05211895 Partnered (Arcus Biosciences)	Unresected, locally advanced NSCLC	860	<ul style="list-style-type: none"> Arm 1: <i>Imfinzi</i> + domvanalimab following chemotherapy/RT Arm 2: <i>Imfinzi</i> + placebo following chemotherapy/RT 	<ul style="list-style-type: none"> Primary endpoint: PFS Secondary endpoint: OS 	<ul style="list-style-type: none"> FPCD: Q1 2022 Data anticipated: >2024
Phase III PACIFIC-9 NCT05221840 Partnered (Innate)	Patients with locally advanced (Stage III), unresectable NSCLC who have not progressed following platinum-based CRT	999	<ul style="list-style-type: none"> Arm 1: <i>Imfinzi</i> + oleclumab Arm 2: <i>Imfinzi</i> + monalizumab + placebo Arm 3: <i>Imfinzi</i> + placebo 	<ul style="list-style-type: none"> Primary endpoint: PFS Secondary endpoints: OS, ORR, DoR, PFS2 and TFST 	<ul style="list-style-type: none"> FPCD: Q2 2022 Data anticipated: >2024
Phase III ADRIATIC NCT03703297	Limited-stage SCLC 1L following platinum-based concurrent chemoradiation therapy	600	<ul style="list-style-type: none"> Arm 1: <i>Imfinzi</i> + <i>Imjudo</i> (4 doses) Arm 2: <i>Imfinzi</i> Arm 3: placebo 	<ul style="list-style-type: none"> Primary endpoints: PFS and OS 	<ul style="list-style-type: none"> FPCD: Q4 2018 Data anticipated: H1 2024



Imfinzi (PD-L1 mAb)

Lung cancer

Trial	Population	Patients	Design	Endpoints	Status
Phase II HUDSON NCT03334617	NSCLC, patients who progressed on an anti-PD-1/PD-L1-containing therapy	521	<ul style="list-style-type: none"> Open-label, biomarker-directed, multi-centre trial Module 1: <i>Imfinzi</i> + <i>Lynparza</i> Module 2: <i>Imfinzi</i> + danvatirsen Module 3: <i>Imfinzi</i> + ceralasertib Module 4: <i>Imfinzi</i> + vistusertib Module 5: <i>Imfinzi</i> + oleclumab Module 6: <i>Imfinzi</i> + <i>Enhertu</i> Module 7: <i>Imfinzi</i> + cediranib Module 8: ceralasertib Module 9: <i>Imfinzi</i> + ceralasertib Module 10: <i>Imfinzi</i> + ceralasertib Module 11: ceralasertib 	<ul style="list-style-type: none"> Primary endpoint: ORR Secondary endpoints: efficacy including OS, PFS, DCR, safety and tolerability and DoR 	<ul style="list-style-type: none"> FPCD: Q1 2018 LPCD: Q3 2023 Data anticipated: >2024
Phase II NeoCOAST NCT03794544	Resectable, early-stage NSCLC	84	<ul style="list-style-type: none"> Arm 1: <i>Imfinzi</i> Arm 2: <i>Imfinzi</i> + oleclumab Arm 3: <i>Imfinzi</i> + monalizumab Arm 4: <i>Imfinzi</i> + danvatirsen 	<ul style="list-style-type: none"> Primary endpoint: major pathological response rate 	<ul style="list-style-type: none"> FPCD: Q1 2019 LPCD: Q1 2021 Data readout: Q1 2022
Phase II NeoCOAST-2 NCT05061550	Early-stage, resectable NSCLC (Stage II to Stage IIIA)	350	<ul style="list-style-type: none"> Open-label trial Arm 1: <i>Imfinzi</i> + oleclumab + platinum doublet chemotherapy Arm 2: <i>Imfinzi</i> + monalizumab + platinum doublet chemotherapy Arm 3: volrustomig + platinum doublet chemotherapy Arm 4: datopotamab deruxtecan + single agent platinum chemotherapy Arm 5: AZD0171 + platinum doublet chemotherapy 	<ul style="list-style-type: none"> Primary endpoints: pCR, safety 	<ul style="list-style-type: none"> FPCD: Q2 2022 Data anticipated: >2024
Phase I/II SCope-D1 NCT04870112	NSCLC, SCLC	18	<ul style="list-style-type: none"> Open-label, multi-centre trial s.c. <i>Imfinzi</i> 	<ul style="list-style-type: none"> Primary endpoints: PK parameters and safety 	<ul style="list-style-type: none"> FPCD: Q4 2021 LPCD: Q2 2022 Data anticipated: H2 2024



Imfinzi (PD-L1 mAb)

Other cancer

Trial	Population	Patients	Design	Endpoints	Status
Phase III POTOMAC NCT03528694	Non-muscle-invasive bladder cancer	1018	<ul style="list-style-type: none"> Arm 1: BCG (induction + maintenance) Arm 2: <i>Imfinzi</i> + BCG (induction only) Arm 3: <i>Imfinzi</i> + BCG (induction + maintenance) 	<ul style="list-style-type: none"> Primary endpoint: DFS 	<ul style="list-style-type: none"> FPCD: Q2 2018 LPCD: Q4 2020 Data anticipated: >2024
Phase III NIAGARA NCT03732677	Muscle-invasive bladder cancer	1063	<ul style="list-style-type: none"> Arm 1: <i>Imfinzi</i> in combination with gemcitabine + cisplatin, <i>Imfinzi</i> maintenance Arm 2: gemcitabine + cisplatin 	<ul style="list-style-type: none"> Co-primary endpoints: pCR and EFS 	<ul style="list-style-type: none"> FPCD: Q4 2018 LPCD: Q3 2021 Data anticipated: >2024
Phase III SAMETA NCT05043090	MET-driven, unresectable and locally advanced or metastatic papillary renal cell carcinoma	200	<ul style="list-style-type: none"> Arm 1: <i>Orpathys</i> + <i>Imfinzi</i> Arm 2: sunitinib Arm 3: <i>Imfinzi</i> monotherapy 	<ul style="list-style-type: none"> Primary endpoint: PFS Secondary endpoints: OS, ORR, DoR and DCR 	<ul style="list-style-type: none"> FPCD: Q4 2021 Data anticipated: >2024
Phase III NILE NCT03682068	1L bladder cancer	1292	<ul style="list-style-type: none"> Arm 1: <i>Imfinzi</i> + <i>Imjudo</i> + SoC Arm 2: <i>Imfinzi</i> + SoC Arm 3: SoC 	<ul style="list-style-type: none"> Primary endpoint: OS 	<ul style="list-style-type: none"> FPCD: Q4 2018 LPCD: Q2 2021 Data anticipated: H1 2024
Phase III VOLGA NCT04960709	Muscle-invasive bladder cancer ineligible to cisplatin	830	<ul style="list-style-type: none"> Arm 1: <i>Imfinzi</i> + <i>Imjudo</i> + enfortumab vedotin Arm 2: <i>Imfinzi</i> + enfortumab vedotin Arm 3: SoC cystectomy 	<ul style="list-style-type: none"> Primary endpoints: safety, EFS and pCR Secondary endpoint: OS 	<ul style="list-style-type: none"> FPCD: Q4 2021 Data anticipated: >2024
Phase II BEGONIA NCT03742102	1L mTNBC	240	<ul style="list-style-type: none"> Arm 1: <i>Imfinzi</i> + paclitaxel Arm 2: <i>Imfinzi</i> + paclitaxel + capivasertib Arm 5: <i>Imfinzi</i> + paclitaxel + oleclumab Arm 6: <i>Imfinzi</i> + <i>Enhertu</i> Arm 7: <i>Imfinzi</i> + datopotamab deruxtecan Arm 8: <i>Imfinzi</i> + datopotamab deruxtecan (PDL1-high) Global trial 	<ul style="list-style-type: none"> Primary endpoints: safety and tolerability Secondary endpoints: ORR, PFS, DoR, OS, PK and ADA 	<ul style="list-style-type: none"> FPCD: Q1 2019 Data anticipated: >2024



Lynparza (PARP inhibitor)

Imfinzi combinations

Trial	Population	Patients	Design	Endpoints	Status
Phase III DUO-O NCT03737643	1L advanced ovarian cancer	1256	<ul style="list-style-type: none"> Non-tBRCAm (tumour BRCA) patients Arm 1: chemotherapy + bevacizumab + <i>Imfinzi</i> placebo followed by bevacizumab + <i>Imfinzi</i> placebo + <i>Lynparza</i> placebo Arm 2: chemotherapy + bevacizumab + <i>Imfinzi</i> followed by bevacizumab + <i>Imfinzi</i> + <i>Lynparza</i> placebo Arm 3: chemotherapy + bevacizumab + <i>Imfinzi</i> followed by bevacizumab + <i>Imfinzi</i> + <i>Lynparza</i> tBRCAm patients chemotherapy + bevacizumab (optional) + <i>Imfinzi</i> followed by bevacizumab (optional) + <i>Imfinzi</i> + <i>Lynparza</i> Global trial 	<ul style="list-style-type: none"> Primary endpoint: PFS Secondary endpoints: OS and PFS2 	<ul style="list-style-type: none"> FPCD: Q1 2019 Data readout: Q2 2023 Primary endpoint met
Phase III DUO-E NCT04269200	1L advanced and recurrent endometrial cancer	699	<ul style="list-style-type: none"> Arm 1: chemotherapy + <i>Imfinzi</i> placebo followed by <i>Imfinzi</i> placebo + <i>Lynparza</i> placebo Arm 2: chemotherapy + <i>Imfinzi</i> followed by <i>Imfinzi</i> + <i>Lynparza</i> placebo Arm 3: chemotherapy + <i>Imfinzi</i> followed by <i>Imfinzi</i> + <i>Lynparza</i> Global trial 	<ul style="list-style-type: none"> Primary endpoint: PFS Secondary endpoints: OS, PFS2, ORR and DoR 	<ul style="list-style-type: none"> FPCD: Q2 2020 Data readout: Q2 2023 Primary endpoint met



Lynparza (PARP inhibitor)

Multiple cancers

Trial	Population	Patients	Design	Endpoints	Status
Phase III OlympiA NCT02032823 Partnered (BIG & NRG Oncology)	gBRCAm adjuvant breast cancer	1836	<ul style="list-style-type: none"> Arm 1: <i>Lynparza</i> BID 12-month duration Arm 2: placebo 12-month duration Global trial in partnership with Breast International Group and National Cancer Institute/NRG Oncology 	<ul style="list-style-type: none"> Primary endpoint: iDFS Secondary endpoints: distant disease-free survival and OS 	<ul style="list-style-type: none"> FPCD: Q2 2014 LPCD: Q2 2019 Data readout: Q1 2021 Primary endpoint met
Phase III MONO-OLA1 NCT04884360	BRCAt advanced ovarian cancer, 1L maintenance	420	<ul style="list-style-type: none"> Arm 1: <i>Lynparza</i> BID 24-month duration Arm 2: placebo BID 24-month duration Global trial – 12 countries 	<ul style="list-style-type: none"> Primary endpoints: PFS (BRCAt HRD+ve) and PFS (BRCAt) Secondary endpoints: OS, TFST and PFS2 	<ul style="list-style-type: none"> FPCD: Q3 2021 Data anticipated: H2 2024



Lynparza (PARP inhibitor)

Other combinations

Trial	Population	Patients	Design	Endpoints	Status
Phase III PROpel NCT03732820	1L metastatic castration-resistant prostate cancer	904	<ul style="list-style-type: none"> Arm 1: <i>Lynparza</i> + abiraterone Arm 2: placebo + abiraterone Global trial (including China) 	<ul style="list-style-type: none"> Primary endpoint: rPFS Secondary endpoints: OS 	<ul style="list-style-type: none"> FPCD: Q4 2018 LPCD: Q3 2022 Data readout: Q3 2021 Primary endpoint met
Phase II LYNK-002 NCT03742895 Partnered (Merck Sharp & Dohme LLC)	HRRm or HRD-positive advanced cancer	390	<ul style="list-style-type: none"> Arm 1: <i>Lynparza</i> Global trial 	<ul style="list-style-type: none"> Primary endpoint: ORR Secondary endpoints: DOR, OS, PFS, AE and Prog by CA-125 	<ul style="list-style-type: none"> FPCD: Q1 2019



Enhertu (trastuzumab deruxtecan, HER2 ADC)

Breast cancer

Trial	Population	Patients	Design	Endpoints	Status
Phase III DESTINY-Breast02 NCT03523585 Partnered (Daiichi Sankyo)	HER2-positive, unresectable and/or metastatic breast cancer pretreated with prior SoC HER2 therapies including trastuzumab emtansine	600	<ul style="list-style-type: none"> Randomised, open-label, parallel assignment Arm 1: <i>Enhertu</i> Arm 2: physician's choice of lapatinib + capecitabine or trastuzumab + capecitabine 	<ul style="list-style-type: none"> Primacy endpoint: PFS Secondary endpoints: OS, ORR, DoR and CBR 	<ul style="list-style-type: none"> FPCD: Q3 2018 LPCD: Q4 2020 Data readout: Q3 2022 Primary endpoint met
Phase III DESTINY-Breast03 NCT03529110 Partnered (Daiichi Sankyo)	HER2-positive, unresectable and/or metastatic breast cancer previously treated with trastuzumab and taxane	524	<ul style="list-style-type: none"> Randomised, open-label, parallel assignment Arm 1: <i>Enhertu</i> Arm 2: ado-trastuzumab emtansine 	<ul style="list-style-type: none"> Primary endpoint: PFS Secondary endpoints: OS, ORR, DoR and CBR 	<ul style="list-style-type: none"> FPCD: Q3 2018 LPCD: Q2 2020 Data readout: Q3 2021 Primary endpoint met
Phase III DESTINY-Breast04 NCT03734029 Partnered (Daiichi Sankyo)	HER2-low, unresectable and/or metastatic breast cancer	557	<ul style="list-style-type: none"> Randomised, open-label, parallel assignment Arm 1: <i>Enhertu</i> Arm 2: physician's choice of SoC chemotherapy (choice of capecitabine, eribulin, gemcitabine, paclitaxel or nab-paclitaxel) 	<ul style="list-style-type: none"> Primary endpoint: PFS Secondary endpoints: OS, DoR and ORR 	<ul style="list-style-type: none"> FPCD: Q4 2018 LPCD: Q4 2020 Data readout: Q1 2022 Primary endpoint met
Phase III DESTINY-Breast05 NCT04622319 Partnered (Daiichi Sankyo)	High-risk HER2-positive with residual invasive breast cancer following neoadjuvant therapy	1600	<ul style="list-style-type: none"> Randomised, open-label, parallel assignment Arm 1: <i>Enhertu</i> Arm 2: ado-trastuzumab emtansine 	<ul style="list-style-type: none"> Primary endpoint: IDFS Secondary endpoints: DFS, OS, DRFI and BMFI 	<ul style="list-style-type: none"> FPCD: Q4 2020 Data anticipated: >2024
Phase III DESTINY-Breast06 NCT04494425 Partnered (Daiichi Sankyo)	HER2-low, HR+ breast cancer with disease progression on endocrine therapy in the metastatic setting	850	<ul style="list-style-type: none"> Randomised, open-label, parallel assignment Arm 1: <i>Enhertu</i> Arm 2: investigator's choice SoC chemotherapy (capecitabine, paclitaxel, nab-paclitaxel) 	<ul style="list-style-type: none"> Primary endpoint: PFS Secondary endpoints: OS, DoR and ORR 	<ul style="list-style-type: none"> FPCD: Q3 2020 Data anticipated: H1 2024
Phase III DESTINY-Breast09 NCT04784715 Partnered (Daiichi Sankyo)	HER2-positive, metastatic breast cancer with no prior therapy for advanced or metastatic disease	1134	<ul style="list-style-type: none"> Randomised, parallel assignment Arm 1: <i>Enhertu</i> + placebo Arm 2: <i>Enhertu</i> + pertuzumab Arm 3: SoC 	<ul style="list-style-type: none"> Primary endpoint: PFS Secondary endpoints: OS, DoR and ORR 	<ul style="list-style-type: none"> FPCD: Q2 2021 Data anticipated: >2024



Enhertu (trastuzumab deruxtecan, HER2 ADC)

Breast cancer

Trial	Population	Patients	Design	Endpoints	Status
Phase III DESTINY-Breast11 NCT05113251 Partnered (Daiichi Sankyo)	High-risk HER2-positive early non-metastatic breast cancer	900	<ul style="list-style-type: none"> Randomised, open-label, parallel assignment Arm 1: <i>Enhertu</i> Arm 2: <i>Enhertu</i> followed by THP Arm 3: doxorubicin and cyclophosphamide followed by THP 	<ul style="list-style-type: none"> Primary endpoint: pCR Secondary endpoints: EFS, IDFS and OS 	<ul style="list-style-type: none"> FPCD: Q4 2021 Data anticipated: >2024
Phase Ib/II DESTINY-Breast07 NCT04538742 Partnered (Daiichi Sankyo)	HER2-positive metastatic breast cancer	245	<ul style="list-style-type: none"> Randomised, open-label, sequential assignment Arm 1: <i>Enhertu</i> Arm 2: <i>Enhertu</i> + <i>Imfinzi</i> Arm 3: <i>Enhertu</i> + pertuzumab Arm 4: <i>Enhertu</i> + paclitaxel Arm 5: <i>Enhertu</i> + <i>Imfinzi</i> + paclitaxel Arm 6: <i>Enhertu</i> + tucatinib 	<ul style="list-style-type: none"> Primary endpoints: AE and SAE Secondary endpoints: ORR, PFS, DoR and OS 	<ul style="list-style-type: none"> FPCD: Q1 2021 Data anticipated: >2024
Phase Ib DESTINY-Breast08 NCT04556773 Partnered (Daiichi Sankyo)	HER2-low metastatic breast cancer	139	<ul style="list-style-type: none"> Non-randomised, open-label parallel assignment Arm 1: <i>Enhertu</i> + capecitabine Arm 2: <i>Enhertu</i> + <i>Imfinzi</i> + paclitaxel Arm 3: <i>Enhertu</i> + capivasertib Arm 4: <i>Enhertu</i> + anastrozole Arm 5: <i>Enhertu</i> + Faslodex 	<ul style="list-style-type: none"> Primary endpoints: AE and SAE Secondary endpoints: ORR, PFS, DoR and OS 	<ul style="list-style-type: none"> FPCD: Q1 2021 Data anticipated: H2 2023



Enhertu (trastuzumab deruxtecan, HER2 ADC)

Gastric cancer

Trial	Population	Patients	Design	Endpoints	Status
Phase III DESTINY-Gastric04 NCT04704934 Partnered (Daiichi Sankyo)	HER2-positive gastric cancer or gastro-esophageal junction adenocarcinoma patients who have progressed on or after a trastuzumab-containing regimen and have not received any additional systemic therapy	490	<ul style="list-style-type: none"> Open-label, randomised, parallel group assignment Arm 1: <i>Enhertu</i> Arm 2: SoC chemotherapy 	<ul style="list-style-type: none"> Primary endpoint: OS Secondary endpoints: ORR, DoR, PFS, DcR and safety 	<ul style="list-style-type: none"> FPCD: Q2 2021 Data anticipated: >2024
Phase II DESTINY-Gastric01 NCT03329690 Partnered (Daiichi Sankyo)	HER2-overexpressing advanced gastric or gastroesophageal junction adenocarcinoma patients who have progressed on two prior treatment regimens	233	<ul style="list-style-type: none"> Randomised, open-label parallel assignment Arm 1: <i>Enhertu</i> Arm 2: SoC chemotherapy Two additional open-label patient cohorts with lower levels of HER2 expression Japan and Korea 	<ul style="list-style-type: none"> Primary endpoint: ORR Secondary endpoints: PFS, OS, DoR, DCR, TTF and range of PK parameters 	<ul style="list-style-type: none"> FPCD: Q4 2017 LPCD: Q2 2019 Data readout: Q1 2020 Primary endpoint met
Phase II DESTINY-Gastric02 NCT04014075 Partnered (Daiichi Sankyo)	HER2-positive gastric cancer that cannot be surgically removed or has spread, in patients who have progressed on or after trastuzumab containing regimen	79	<ul style="list-style-type: none"> Open-label, single group assignment <i>Enhertu</i> Western population 	<ul style="list-style-type: none"> Primary endpoint: ORR Secondary endpoints: PFS, ORR, OS and DoR 	<ul style="list-style-type: none"> FPCD: Q4 2019 LPCD: Q4 2020 Data readout: Q2 2021 Primary endpoint met
Phase II DESTINY-Gastric06 NCT04989816 Partnered (Daiichi Sankyo)	HER2-positive gastric cancer or gastro-esophageal junction adenocarcinoma patients who have progressed on two prior treatment regimens	95	<ul style="list-style-type: none"> Open-label, single group assignment <i>Enhertu</i> China only 	<ul style="list-style-type: none"> Primary endpoint: ORR Secondary endpoints: PFS, ORR, DCR, OS, DoR and safety 	<ul style="list-style-type: none"> FPCD: Q3 2021 Data readout: Q3 2023
Phase Ib/II DESTINY-Gastric03 NCT04379596 Partnered (Daiichi Sankyo)	HER2-overexpressing gastric or gastroesophageal junction cancer	357	<ul style="list-style-type: none"> Open-label, parallel assignment Part 1: to determine recommended Phase II combination dose 5 Arms combining <i>Enhertu</i> with SoC chemotherapies (5-FU, capecitabine, oxaliplatin) and/or durvalumab Part 2: to assess efficacy of the selected combinations Arm 2A: standard chemotherapy Arm 2B: <i>Enhertu</i> monotherapy Arm 2C: <i>Enhertu</i> with chemotherapy Arm 2D: <i>Enhertu</i> with chemotherapy and pembrolizumab Arm 2E: <i>Enhertu</i> and pembrolizumab Arm 3A: <i>Enhertu</i>, FP and volrustomig Arm 3B: <i>Enhertu</i>, FP and volrustomig 	<ul style="list-style-type: none"> Primary endpoint (Part 1): safety Primary endpoint (Part 2): ORR Secondary endpoints: DoR, DCR, PFS, OS, PK parameters and presence of ADAs 	<ul style="list-style-type: none"> FPCD: Q2 2020 Data anticipated: >2024



Enhertu (trastuzumab deruxtecan, HER2 ADC)

Other cancers

Trial	Population	Patients	Design	Endpoints	Status
Phase III DESTINY-Lung04 NCT05048797 Partnered (Daiichi Sankyo)	HER2-mutated, unresectable, locally advanced/metastatic NSCLC	264	<ul style="list-style-type: none"> Randomised, parallel group assignment Arm 1: <i>Enhertu</i> Arm 2: SoC (platinum, pemetrexed and pembrolizumab) 	<ul style="list-style-type: none"> Primary endpoint: PFS Secondary endpoints: OS, CNS-PFS, PFS (INV), ORR, DoR, safety, PK parameters, ADA, PRO-tolerability and PRO- pulmonary symptoms 	<ul style="list-style-type: none"> FPCD: Q4 2021 Data anticipated: >2024
Phase II DESTINY-Lung02 NCT04644237 Partnered (Daiichi Sankyo)	HER2-mutated, unresectable and/or metastatic NSCLC	152	<ul style="list-style-type: none"> Randomised, parallel group assignment Arm 1: <i>Enhertu</i> 6.4mg/kg Arm 2: <i>Enhertu</i> 5.4mg/kg 	<ul style="list-style-type: none"> Primary endpoint: ORR Secondary endpoints: DoR, DCR, PFS, OS and PK parameters 	<ul style="list-style-type: none"> FPCD: Q1 2021 Data readout: Q1 2023 Primary endpoint met
Phase II DESTINY-PanTumor02 NCT04482309 Partnered (Daiichi Sankyo)	HER2-expressing tumours	468	<ul style="list-style-type: none"> Non-randomised, single group assignment <i>Enhertu</i> 	<ul style="list-style-type: none"> Primary endpoint: ORR Secondary endpoints: DoR, DCR, PFS and OS 	<ul style="list-style-type: none"> FPCD: Q4 2020 Data readout: Q3 2023
Phase II DESTINY-PanTumor01 NCT04639219 Partnered (Daiichi Sankyo)	HER2-mutated tumours	102	<ul style="list-style-type: none"> Non-randomised, single group assignment <i>Enhertu</i> 	<ul style="list-style-type: none"> Primary endpoint: ORR Secondary endpoints: DoR, DCR, PFS and PK parameters 	<ul style="list-style-type: none"> FPCD: Q1 2021 Data readout: Q2 2023
Phase II DESTINY-CRC02 NCT04744831 Partnered (Daiichi Sankyo)	HER2-overexpressing advanced or metastatic colorectal cancer	120	<ul style="list-style-type: none"> Randomised, parallel group assignment Arm 1: <i>Enhertu</i> 6.4mg/kg Arm 2: <i>Enhertu</i> 5.4mg/kg 	<ul style="list-style-type: none"> Primary endpoint: ORR Secondary endpoints: ORR, PFS, OS, DoR, DCR and PK parameters 	<ul style="list-style-type: none"> FPCD: Q1 2021 Data readout: Q1 2023 Primary endpoint met



Enhertu (trastuzumab deruxtecan, HER2 ADC)

Other cancers

Trial	Population	Patients	Design	Endpoints	Status
Phase Ib DESTINY-Lung03 NCT04686305 Partnered (Daiichi Sankyo)	HER2-over-expressing, unresectable and/or metastatic NSCLC	168	<ul style="list-style-type: none"> Non-randomised, parallel group assignment Part 1: to determine recommended combination dose 3 Arms combine <i>Enhertu</i> with SoC chemotherapies (cisplatin, carboplatin or pemetrexed) and <i>Imfinzi</i>; Arm 1D: <i>Enhertu</i> monotherapy arm Part 2: to assess efficacy of the selected combinations with chemotherapies (cisplatin, carboplatin or pemetrexed) and <i>Imfinzi</i> not initiated Part 3 (2 arms): dose confirmation to assess safety and efficacy with volrustomig and volrustomig and chemotherapy (carboplatin) 	<ul style="list-style-type: none"> Primary endpoint: safety and RP2D Secondary endpoints: ORR, DoR, DCR, PFS, OS and PK parameters 	<ul style="list-style-type: none"> FPCD: Q4 2021 Data anticipated: >2024
Phase Ib U106 NCT04042701 Partnered (Daiichi Sankyo)	HER2-expressing locally advanced/metastatic breast or NSCLC	115	<ul style="list-style-type: none"> Non-randomised, parallel group assignment <i>Enhertu</i> + pembrolizumab Global trial – 2 countries 	<ul style="list-style-type: none"> Primary endpoints: DLT and ORR Secondary endpoints: DoR, DCR, PFS, TTR and OS 	<ul style="list-style-type: none"> FPCD: Q2 2020 Data anticipated: H2 2024



Calquence (BTK inhibitor)

Blood cancers

Trial	Population	Patients	Design	Endpoints	Status
Phase III AMPLIFY (ACE-CL-311) NCT03836261	Previously untreated CLL	981	<ul style="list-style-type: none"> Arm 1; <i>Calquence</i> + venetoclax Arm 2: <i>Calquence</i> + venetoclax + obinutuzumab Arm 3: FCR or BR 	<ul style="list-style-type: none"> Primary endpoint: IRC PFS (Arm 1 vs. Arm 3) Secondary endpoints: IRC PFS (Arm 2 vs. Arm 3) and INV PFS (Arm 1 vs. Arm 3; Arm 2 vs. Arm 3) 	<ul style="list-style-type: none"> FPCD: Q1 2019 Data anticipated: >2024
Phase III ASCEND (ACE-CL-309) NCT02970318	R/R CLL	306	<ul style="list-style-type: none"> Arm 1: <i>Calquence</i> Arm 2: rituximab + idelalisib or bendamustine (investigator's choice) 	<ul style="list-style-type: none"> Primary endpoint: IRC assessed PFS (Arm 1 vs. Arm 2) Secondary endpoints: INV-assessed ORR, OS, DoR and PROs 	<ul style="list-style-type: none"> FPCD: Q4 2017 Data readout: Q2 2019 Primary endpoint met
Phase III ECHO (ACE-LY-308) NCT02972840	Previously untreated MCL	634	<ul style="list-style-type: none"> Arm 1: <i>Calquence</i> + bendamustine + rituximab Arm 2: bendamustine + rituximab 	<ul style="list-style-type: none"> Primary endpoint: PFS by Lugano Classification for NHL Secondary endpoints: IA, PFS, ORR, DoR, time to response and OS 	<ul style="list-style-type: none"> FPCD: Q2 2017 Data anticipated: >2024
Phase III ESCALADE NCT04529772	DLBCL	600	<ul style="list-style-type: none"> <i>Calquence</i> + rituximab, cyclophosphamide, doxorubicin, vincristine and prednisone 	<ul style="list-style-type: none"> Primary endpoint: PFS 	<ul style="list-style-type: none"> FPCD: Q1 2021 Data anticipated: >2024
Phase III NCT04075292	Untreated CLL	155	<ul style="list-style-type: none"> Arm 1: <i>Calquence</i> Arm 2: chlorambucil + rituximab 	<ul style="list-style-type: none"> Primary endpoint: PFS Secondary endpoints: ORR and DoR 	<ul style="list-style-type: none"> FPCD: Q1 2020 Data anticipated: H2 2024
Phase Ib ACE-LY-106 NCT02717624	MCL	61	<ul style="list-style-type: none"> <i>Calquence</i> in combination with bendamustine and rituximab Arm 1: treatment naïve Arm 2: R/R Arm 3: treatment naïve: <i>Calquence</i> + venetoclax + rituximab 	<ul style="list-style-type: none"> Primary endpoint: safety 	<ul style="list-style-type: none"> FPCD: Q2 2016 LPCD: Q2 2022 Data readout: Q1 2023
Phase I ACE-LY-003 NCT02180711	R/R follicular lymphoma	89	<ul style="list-style-type: none"> Arm 1: <i>Calquence</i> Arm 2: <i>Calquence</i> + rituximab Arm 3: <i>Calquence</i> + rituximab + lenolidomide 	<ul style="list-style-type: none"> Primary endpoint: safety 	<ul style="list-style-type: none"> FPCD: Q1 2015 Data anticipated: H1 2024



Calquence (BTK inhibitor)

Blood cancers

Approved medicines

Late-stage development

Early development

Oncology

CVRM

R&I

Other

V&I

Rare Disease

Trial	Population	Patients	Design	Endpoints	Status
Phase I ACE-CL-003 NCT02296918	CLL/SLL/PLL	114	<ul style="list-style-type: none">• <i>Calquence</i> + obinutuzumab• Arm 1: R/R• Arm 2: treatment naïve• <i>Calquence</i> + venetoclax + rituxumab• Arm 3: R/R• Arm 4: treatment naïve	<ul style="list-style-type: none">• Primary endpoints: safety and ORR• Secondary endpoints: PD, PFS, TTNT and OS	<ul style="list-style-type: none">• FPCD: Q4 2014• Data readout: Q1 2022



Orpathys (savolitinib, MET inhibitor)

NSCLC and other cancers

Trial	Population	Patients	Design	Endpoints	Status
Phase III NCT04923945 Partnered (HUTCHMED)	Locally advanced or metastatic NSCLC patients with MET exon 14 mutations without EGFR, ALK and ROS1 mutations progressing on platinum chemotherapy and are treatment naïve to c-MET therapy or did not receive prior drug therapy for advanced tumours	163	<ul style="list-style-type: none"> Single-arm trial <i>Orpathys</i> 	<ul style="list-style-type: none"> Primary endpoint: ORR 	<ul style="list-style-type: none"> FPCD: Q3 2021 Data anticipated: H2 2024
Phase II NCT04923932 Partnered (HUTCHMED)	Locally advanced or metastatic gastric cancer and esophagogastric junction adenocarcinoma patients with MET gene amplifications	75	<ul style="list-style-type: none"> Single-arm, multi-cohort, multi-centre, open-label trial <i>Orpathys</i> 	<ul style="list-style-type: none"> Primary endpoint: ORR Secondary endpoints: PFS and safety 	<ul style="list-style-type: none"> FPCD: Q3 2021 Data anticipated: H2 2024



capivasertib (AKT inhibitor)

Breast cancer and prostate cancer

Trial	Population	Patients	Design	Endpoints	Status
Phase III CAPitello-290 NCT03997123	Locally advanced or metastatic TNBC	924	<ul style="list-style-type: none"> • Double-blind, randomised, comparative trial • Arm 1: capivasertib + paclitaxel • Arm 2: placebo + paclitaxel 	<ul style="list-style-type: none"> • Primary endpoint: OS 	<ul style="list-style-type: none"> • FPCD: Q3 2019 • Data anticipated: H1 2024
Phase III CAPitello-291 NCT04305496	2L+ AI-resistant locally advanced (inoperable) or metastatic HR+/HER2- breast cancer	834	<ul style="list-style-type: none"> • Double-blind, randomised, comparative trial • Arm 1: capivasertib + <i>Faslodex</i> • Arm 2: placebo + <i>Faslodex</i> 	<ul style="list-style-type: none"> • Primary endpoint: PFS 	<ul style="list-style-type: none"> • FPCD: Q2 2020 • Data readout: Q4 2022 • Both primary endpoints met
Phase III CAPitello-281 NCT04493853	De novo PTEN deficient metastatic hormone sensitive prostate cancer	1000	<ul style="list-style-type: none"> • Double-blind, randomised, comparative trial • Arm 1: capivasertib + abiraterone • Arm 2: placebo + abiraterone 	<ul style="list-style-type: none"> • Primary endpoint: rPFS 	<ul style="list-style-type: none"> • FPCD: Q3 2020 • Data anticipated: >2024
Phase Ib/III CAPitello-292 NCT04862663	1L triplet in early relapse/endocrine-resistant locally advanced (inoperable) or metastatic HR+/HER2- breast cancer	700	<ul style="list-style-type: none"> • Double-blind, randomised, comparative trial • Arm 1: capivasertib + palbociclib + <i>Faslodex</i> • Arm 2: placebo + palbociclib + <i>Faslodex</i> 	<ul style="list-style-type: none"> • Primary endpoint: PFS 	<ul style="list-style-type: none"> • FPCD (Phase Ib): Q2 2021 • FPCD (Phase III): Q4 2023 • Data anticipated: >2024
Phase III CAPitello-280 NCT05348577	mCRPC prostate cancer	790	<ul style="list-style-type: none"> • Double-blind, randomised, comparative trial • Arm 1: capivasertib + docetaxel • Arm 2: placebo + docetaxel 	<ul style="list-style-type: none"> • Primary endpoint: OS 	<ul style="list-style-type: none"> • FPCD: Q2 2022 • Data anticipated: >2024



datopotamab deruxtecan (TROP2 ADC)

Breast cancer

Trial	Population	Patients	Design	Endpoints	Status
Phase III TROPION-Breast01 NCT05104866 Partnered (Daiichi Sankyo)	Inoperable or metastatic HR+ HER2-breast cancer after treatment with one or two prior lines of systemic chemotherapy	733	<ul style="list-style-type: none"> Open-label, randomised trial Arm 1: datopotamab deruxtecan Arm 2: investigator's choice SoC chemotherapy (eribulin, vinorelbine, capecitabine, gemcitabine) 	<ul style="list-style-type: none"> Primary endpoints: PFS (BICR) and OS Secondary endpoints: ORR, DoR, PFS (Inv), DCR, PK parameters and ADA 	<ul style="list-style-type: none"> FPCD: Q4 2021 LPCD: Q4 2022 Data readout: Q3 2023
Phase III TROPION-Breast02 NCT05374512 Partnered (Daiichi Sankyo)	Locally recurrent inoperable or metastatic TNBC	600	<ul style="list-style-type: none"> Open-label, randomised trial Arm 1: datopotamab deruxtecan Arm 2: investigator's choice of chemotherapy (paclitaxel, nab-paclitaxel, carboplatin, capecitabine, eribulin mesylate) Global trial 	<ul style="list-style-type: none"> Primary endpoints: PFS (BICR) and OS Secondary endpoints: PFS (Inv), ORR, DoR, PK parameters and ADA 	<ul style="list-style-type: none"> FPCD: Q2 2022 Data anticipated: H2 2024
Phase III TROPION-Breast03 NCT05629585 Partnered (Daiichi Sankyo)	Stage I-III TNBC without pathological complete response following neoadjuvant therapy	1075	<ul style="list-style-type: none"> Open-label, randomised Arm 1: datopotamab deruxtecan + <i>Imfinzi</i> Arm 2: datopotamab deruxtecan Arm 3: investigator's choice of therapy (capecitabine, pembrolizumab, or capecitabine + pembrolizumab) Global trial 	<ul style="list-style-type: none"> Primary endpoint: iDFS Secondary endpoints: DDFS, OS, PK and ADA 	<ul style="list-style-type: none"> FPCD: Q4 2022 Data anticipated: >2024



datopotamab deruxtecan (TROP2 ADC)

NSCLC

Trial	Population	Patients	Design	Endpoints	Status
Phase III TROPION-Lung01 NCT04656652 Partnered (Daiichi Sankyo)	Previously treated advanced or metastatic NSCLC with or without actionable genomic alterations	590	<ul style="list-style-type: none"> Randomised, open-label, parallel assignment Arm 1: datopotamab deruxtecan Arm 2: docetaxel Global trial 	<ul style="list-style-type: none"> Primary endpoints: PFS and OS Secondary endpoints: ORR, DoR, TTR, DCR, PK parameters and ADA 	<ul style="list-style-type: none"> FPCD: Q1 2021 LPD: Q4 2022 Data readout: Q3 2023 Dual primary endpoint met (PFS)
Phase III TROPION-Lung08 NCT05215340 Partnered (Daiichi Sankyo)	Treatment-naïve patients with PD-L1-high advanced or metastatic NSCLC without actionable genomic alterations	740	<ul style="list-style-type: none"> Randomised, open-label Arm 1: datopotamab deruxtecan + pembrolizumab Arm 2: pembrolizumab Global trial 	<ul style="list-style-type: none"> Primary endpoints: PFS and OS 	<ul style="list-style-type: none"> FPCD: Q1 2022 Data anticipated: >2024
Phase III TROPION-Lung07 NCT05555732 Partnered (Daiichi Sankyo)	1L patients with PD-L1 TPS <50% and advanced or metastatic NSCLC without actionable genomic alterations	975	<ul style="list-style-type: none"> Randomised, open-label Arm 1: datopotamab deruxtecan + pembrolizumab + platinum chemotherapy Arm 2: datopotamab deruxtecan + pembrolizumab Arm 3: pembrolizumab + pemetrexed + platinum chemotherapy Global trial 	<ul style="list-style-type: none"> Primary endpoints: PFS and OS 	<ul style="list-style-type: none"> FPCD: Q1 2023 Data anticipated: >2024
Phase III AVANZAR NCT05687266	1L NSCLC	1000	<ul style="list-style-type: none"> Arm 1: carboplatin + datopotamab deruxtecan + <i>Imfinzi</i> Arm 2: pembrolizumab Global trial 	<ul style="list-style-type: none"> Co-primary endpoints: OS and PFS in TROP2 biomarker-positive 	<ul style="list-style-type: none"> FPCD: Q1 2023 Data anticipated: >2024



datopotamab deruxtecan (TROP2 ADC)

NSCLC

Approved medicines

Late-stage development

Early development

Oncology

CVRM

R&I

Other

V&I

Rare Disease

Trial	Population	Patients	Design	Endpoints	Status
Phase II TROPION-Lung05 NCT04484142 Partnered (Daiichi Sankyo)	Advanced or metastatic NSCLC with actionable genomic alterations and progressed on or after kinase inhibitor therapy and platinum-based chemotherapy	137	<ul style="list-style-type: none"> Single-arm, open-label datopotamab deruxtecan Global trial 	<ul style="list-style-type: none"> Primary endpoint: ORR Secondary endpoints: DOR, PFS, OS, safety, PK parameters and ADA 	<ul style="list-style-type: none"> FPCD: Q1 2021 LPCD: Q1 2022 Data anticipated: H2 2024
Phase I TROPION-Lung02 NCT04526691 Partnered (Daiichi Sankyo)	Advanced or metastatic NSCLC	145	<ul style="list-style-type: none"> Open-label, two-part (dose escalation and dose expansion), sequential assignment datopotamab deruxtecan + pembrolizumab +/- platinum chemotherapy Global trial – US, Japan, Italy, Spain and Taiwan 	<ul style="list-style-type: none"> Primary endpoints: DLT and safety Secondary endpoints: ORR, DOR, PFS, OS, PK parameters and ADA 	<ul style="list-style-type: none"> FPCD: Q4 2020 LPCD: Q2 2023 Data anticipated: H1 2024
Phase I TROPION-Lung04 NCT04612751 Partnered (Daiichi Sankyo)	Advanced or metastatic NSCLC	232	<ul style="list-style-type: none"> Open-label, two-part (dose escalation, dose expansion), sequential assignment datopotamab deruxtecan + <i>Imfinzi</i> +/- platinum chemotherapy Cohort 1 & 2: datopotamab deruxtecan + <i>Imfinzi</i> Cohort 3 & 4: datopotamab deruxtecan + <i>Imfinzi</i> + carboplatin Cohort 5 & 6: datopotamab deruxtecan + rilvegostomig Cohort 7 & 8: datopotamab deruxtecan + rilvegostomig + carboplatin Cohort 9 & 10: datopotamab deruxtecan + volrustomig + carboplatin Cohort 11: datopotamab deruxtecan + volrustomig Global trial 	<ul style="list-style-type: none"> Primary endpoints: DLT and safety Secondary endpoints: ORR, DOR, PFS, OS, PK parameters and ADA 	<ul style="list-style-type: none"> FPCD: Q1 2021 Data anticipated: >2024



datopotamab deruxtecan (TROP2 ADC)

Other cancers

Trial	Population	Patients	Design	Endpoints	Status
Phase II TROPION-PanTumor03 NCT05489211 Partnered (Daiichi Sankyo)	Endometrial cancer, gastric cancer, mCRPC, ovarian cancer, CRC, bladder cancer and BTC	531	<ul style="list-style-type: none"> Sub-study 1 (endometrial cancer); Sub-study 1a: datopotamab deruxtecan monotherapy Sub-study 1b: datopotamab deruxtecan + <i>Imfinzi</i>, Sub-study 1c: datopotamab deruxtecan + saruparib Sub-study 1d: datopotamab deruxtecan + <i>Imfinzi</i> + saruparib Sub-study 2 (gastric cancer); Sub-study 2a: datopotamab deruxtecan + capecitabine Sub-study 2b: datopotamab deruxtecan + 5-fluorouracil Sub-study 2c: datopotamab deruxtecan + chemotherapy (capecitabine or 5-FU) + nivolumab Sub-study 3 (mCRPC); Sub-study 3a: datopotamab deruxtecan Sub-study 3b: datopotamab deruxtecan + saruparib Sub-study 3c: datopotamab deruxtecan + prednisone/prednisolone Sub-study 4 (ovarian cancer) Sub-study 4a: datopotamab deruxtecan Sub-study 4b Arm1: datopotamab deruxtecan + carboplatin Arm2: datopotamab deruxtecan + saruparib Sub-study 5 (CRC) Sub-study 5a: datopotamab deruxtecan Sub-study 5b Arm 1: datopotamab deruxtecan + 5-FU + leucovorin + bevacizumab Arm 2: datopotamab deruxtecan + capecitabine + bevacizumab Sub-study 6 (bladder) Arm 1: 1L cis-ineligible/2L datopotamab deruxtecan + volrustomig Arm 2: 1L cis-ineligible/2L datopotamab deruxtecan + rilvegostomig Sub-study 7 (BTC) Arm 7a: TROP2+ 2L+ datopotamab deruxtecan 	<ul style="list-style-type: none"> Primary endpoints: ORR and safety 	<ul style="list-style-type: none"> FPCD: Q3 2022 Data anticipated: >2024



datopotamab deruxtecan (TROP2 ADC)

Other cancers

Trial	Population	Patients	Design	Endpoints	Status
Phase I/II TROPION-PanTumor02 NCT05460273 Partnered (Daiichi Sankyo)	NSCLC and TNBC and other solid tumours in Chinese patients	119	<ul style="list-style-type: none"> Single-arm, multi-cohort study with no blinding datopotamab deruxtecan China only 	<ul style="list-style-type: none"> Primary endpoint: ORR Secondary endpoints: DoR, DCR, BOR, TTR PFS and OS 	<ul style="list-style-type: none"> FPCD: Q3 2022 LPD: Q2 2023 Data anticipated: H2 2023
Phase I TROPION-PanTumor01 NCT03401385 Partnered (Daiichi Sankyo)	Subjects with advanced solid tumours: NSCLC, TNBC, HR+ breast cancer, HER2-negative gastric/GEJ, oesophageal, urothelial, SCLC, CRPC, PDAC, HNSCC, HR+ HER2-low breast cancer and HER2+ breast cancer	890	<ul style="list-style-type: none"> Open-label, two-part (dose escalation, dose expansion), sequential assignment datopotamab deruxtecan US and Japan 	<ul style="list-style-type: none"> Primary endpoints: DLT and safety Secondary endpoints: PK parameters, anti-tumour activity and ADA 	<ul style="list-style-type: none"> FPCD: Q1 2018 Data anticipated: H2 2024



camizestrant (AZD9833, next-generation oral SERD)

Breast cancer

Trial	Population	Patients	Design	Endpoints	Status
Phase III SERENA-4 NCT04711252	HR+ HER2- advanced breast cancer	1342	<ul style="list-style-type: none"> Randomised, double-blind, comparative trial Arm 1: camizestrant + palbociclib Arm 2: anastrozole + palbociclib 	<ul style="list-style-type: none"> Primary endpoint: PFS Secondary endpoints: OS and PFS2 	<ul style="list-style-type: none"> FPCD: Q1 2021 Data anticipated: >2024
Phase III SERENA-6 NCT04964934	HR+ HER2- advanced breast cancer	300	<ul style="list-style-type: none"> Randomised, double-blind, comparator trial Arm 1: camizestrant + palbociclib or abemaciclib or ribociclib Arm 2: anastrozole or letrozole + palbociclib or abemaciclib or ribociclib 	<ul style="list-style-type: none"> Primary endpoint: PFS Secondary endpoint: OS and PFS2 	<ul style="list-style-type: none"> FPCD: Q3 2021 Data anticipated: >2024
Phase III CAMBRIA-1 NCT05774951	ER+/HER2- early breast cancer patients who completed definitive locoregional therapy and standard adjuvant ET for at least 2 years and up to 5 years	4300	<ul style="list-style-type: none"> Arm 1: continue standard ET of investigator's choice Arm 2: camizestrant Global trial – 39 countries 	<ul style="list-style-type: none"> Primary endpoint: IBCFS Secondary endpoints: IDFS, DRFS and OS 	<ul style="list-style-type: none"> FPCD: Q1 2023 Data anticipated: >2024
Phase III CAMBRIA-2 NCT05952557	ER+/HER2- early breast cancer with intermediate-high or high risk of recurrence that has completed definitive locoregional therapy and have no evidence of disease	5500	<ul style="list-style-type: none"> Arm A: standard endocrine therapy of investigator's choice (aromatase inhibitors [exemestane, letrozole, anastrozole] or tamoxifen) ± abemaciclib Arm B: camizestrant ± abemaciclib Global study – 43 countries 	<ul style="list-style-type: none"> Primary endpoint: IBCFS Secondary endpoints: IDFS, DRFS and OS 	<ul style="list-style-type: none"> FPCD: Q4 2023 Data anticipated: >2024
Phase II SERENA-2 NCT04214288	HR+ advanced breast cancer	240	<ul style="list-style-type: none"> Randomised, open-label, parallel-group, multi-centre trial Arm 1: camizestrant (75mg) Arm 2: camizestrant (150mg) Arm 3: camizestrant (300mg) Arm 4: Faslodex 	<ul style="list-style-type: none"> Primary endpoint: PFS 	<ul style="list-style-type: none"> FPCD: Q2 2020 LPCD: Q3 2021 Data readout: Q4 2022 Primary endpoint met at 75mg and 150mg doses
Phase II SERENA-3 NCT04588298	HR+ HER2- early breast cancer	135	<ul style="list-style-type: none"> Randomised, open-label, parallel-group, multi-centre trial camizestrant 	<ul style="list-style-type: none"> Primary endpoint: change in ER expression between pre- and on-treatment tumour biopsies 	<ul style="list-style-type: none"> FPCD: Q4 2020 LPCD: Q2 2023 Data readout: Q3 2023



camizestrant (AZD9833, next-generation oral SERD)

Breast cancer

Trial	Population	Patients	Design	Endpoints	Status
Phase I NCT04818632	HR+ HER2- metastatic breast cancer in Chinese patients	30	<ul style="list-style-type: none"> Dose escalation: camizestrant Dose expansion: Cohort 1: camizestrant Cohort 2: camizestrant + palbociclib Cohort 3: camizestrant + everolimus China only 	<ul style="list-style-type: none"> Primary endpoints: safety and tolerability, PK parameters Secondary endpoint: anti-tumour activity 	<ul style="list-style-type: none"> FPCD: Q1 2021 LPCD: Q1 2023 Data anticipated: H2 2023
Phase I NCT04541433	HR+ HER2- advanced breast cancer	18	<ul style="list-style-type: none"> Open-label trial camizestrant Japan only 	<ul style="list-style-type: none"> Primary endpoints: safety and tolerability Secondary endpoint: PK parameters 	<ul style="list-style-type: none"> FPCD: Q4 2020 LPCD: Q1 2022 Data readout: Q1 2023
Phase I SERENA-1 NCT03616587	HR+ HER2- advanced breast cancer	386	<ul style="list-style-type: none"> Escalation phase: open-label multi-centre trial Cohort 1: camizestrant Cohort 2: camizestrant + palbociclib, everolimus, abemeciclib (+/- anastrozole), capivasertib, ribociclib (+/- anastrozole) or anastrozole Expansion phase: randomised expansion cohort(s) Cohort 1: camizestrant Cohort 2: camizestrant + palbociclib, everolimus, abemeciclib (+/- anastrozole), capivasertib, ribociclib (+/- anastrozole) or anastrozole 	<ul style="list-style-type: none"> Primary endpoints: safety and tolerability Secondary endpoints: PK parameters and anti-tumour activity 	<ul style="list-style-type: none"> FPCD: Q4 2018 Data anticipated: H2 2024



ceralasertib (AZD6738, ATR inhibitor)

Multiple cancers

Trial	Population	Patients	Design	Endpoints	Status
Phase III LATIFY NCT05450692	Post-IO NSCLC	580	<ul style="list-style-type: none"> Double-arm randomised: Arm 1: ceralasertib + <i>Imfinzi</i> Arm 2: docetaxel 	<ul style="list-style-type: none"> Primary endpoint: OS Secondary endpoint: PFS, ORR, DoR, TTR, DCR, PFS2 and TTD 	<ul style="list-style-type: none"> FPCD: Q4 2022 Data anticipated: >2024
Phase II MONETTE NCT05061134	2L+ post-IO melanoma	180	<ul style="list-style-type: none"> Double-armed randomised and biopsy sub-study Arm 1: ceralasertib + <i>Imfinzi</i> Arm 2: ceralasertib Arm 3: ceralasertib (biopsy sub-study) 	<ul style="list-style-type: none"> Primary endpoint: ORR Secondary endpoints: DoR, TTR, PFS, OS, safety and biomarkers 	<ul style="list-style-type: none"> FPCD: Q3 2022 LPCD: Q3 2023 Trial discontinued due to futility
Phase I/II NCT02264678	Solid tumours	330	<ul style="list-style-type: none"> Module 1: ceralasertib + carboplatin Module 2: ceralasertib dose escalation, ceralasertib + <i>Lynparza</i> Module 3: ceralasertib + <i>Imfinzi</i> Module 4: ceralasertib monotherapy + <i>Lynparza</i> + <i>Imfinzi</i> (food effect/QT) Module 5: ceralasertib + saruparib Global trial – North America, Europe and South Korea 	<ul style="list-style-type: none"> Primary endpoints: safety and tolerability, efficacy and PK parameters 	<ul style="list-style-type: none"> FPCD: Q4 2014 Data anticipated: >2024



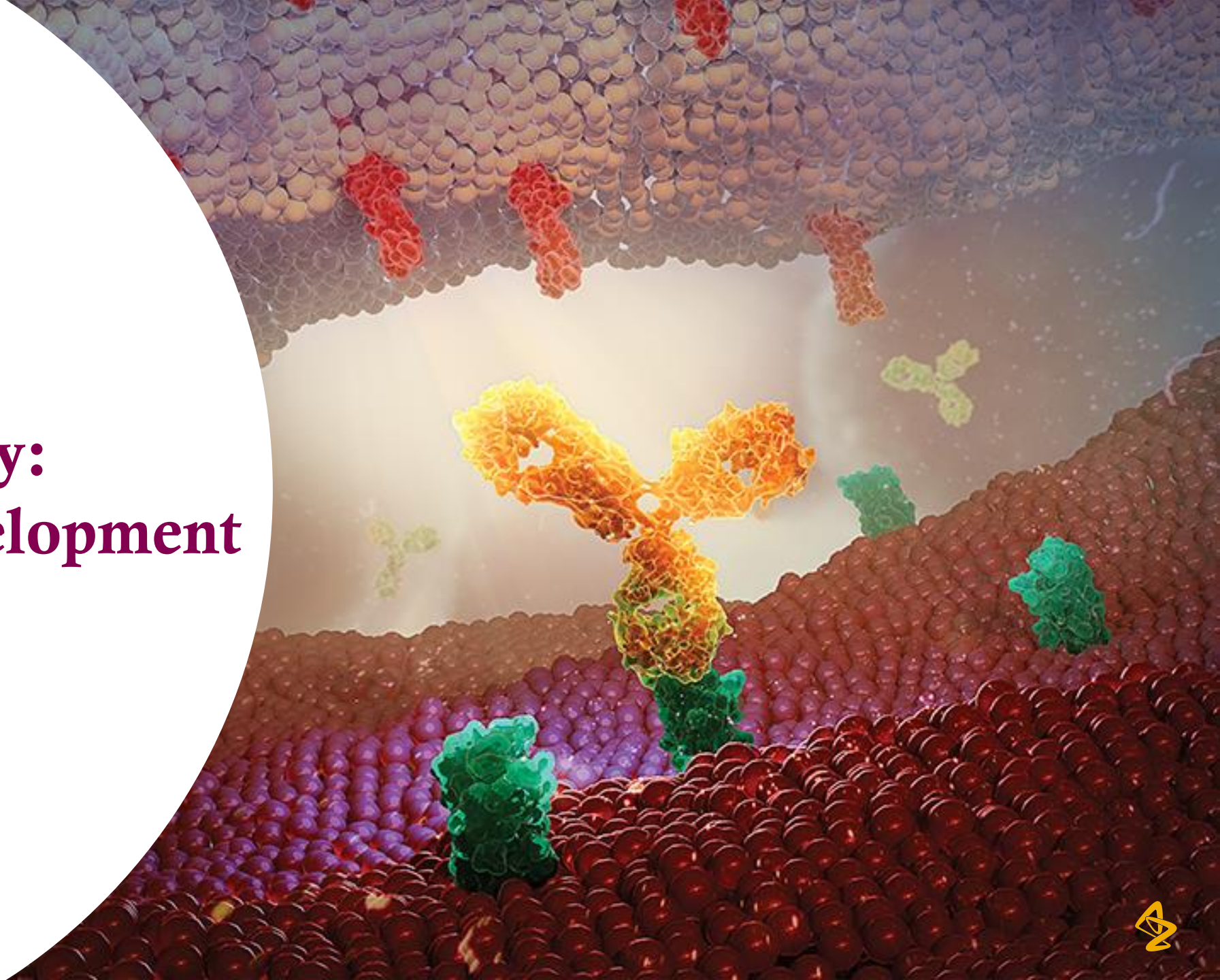
volrustomig (MEDI5752, PD-1/CTLA-4 bispecific mAb)

Solid tumours

Trial	Population	Patients	Design	Endpoints	Status
Phase III eVOLVE-Cervical NCT06079671	High-risk locally advanced cervical cancer with no progression following platinum-based CCRT	1000	<ul style="list-style-type: none"> Randomised, double-blind, placebo-controlled, multi-centre trial Arm 1: volrustomig Arm 2: placebo 	<ul style="list-style-type: none"> Primary endpoints: PFS Secondary endpoint: OS 	<ul style="list-style-type: none"> FPCD: Q4 2023 Data anticipated: >2024
Phase III eVOLVE-Lung02 NCT05984277	1L mNSCLC with PD-L1 <50%	900	<ul style="list-style-type: none"> Double-arm randomized, open-label trial Arm 1: volrustomig + chemotherapy Arm 2: pembrolizumab + chemotherapy 	<ul style="list-style-type: none"> Primary endpoints: PFS and OS 	<ul style="list-style-type: none"> Initiating Data anticipated: >2024
Phase Ib NCT04522323	Advanced renal cell carcinoma	179	<ul style="list-style-type: none"> Open-label, dose escalation and dose expansion trial Arm 1: volrustomig and axitinib Arm 2: volrustomig and lenvatanib 	<ul style="list-style-type: none"> Primary endpoints (escalation): safety, MTD, RP2D, tolerability and anti-tumour activity of combination (ORR) Secondary endpoints: PK parameters, ADA and anti-tumour activity (PFS, OR, DoR, DCR, TTR, OS) 	<ul style="list-style-type: none"> FPCD: Q3 2020 Data anticipated: >2024
Phase I NCT03530397	Advanced solid tumours	396	<ul style="list-style-type: none"> Open-label, dose escalation and dose expansion trial Dose escalation: volrustomig i.v. Dose expansion: volrustomig i.v. as monotherapy and in combination with chemotherapy Arm 1: volrustomig i.v. Arm 2: volrustomig i.v., pemetrexed and carboplatin Arm 3: pembrolizumab, pemetrexed and carboplatin Arm 4: volrustomig i.v., taxane (paclitaxel or nab-paclitaxel) and carboplatin 	<ul style="list-style-type: none"> Primary endpoints (escalation): safety and tolerability, MTD, OBD and HPDD Primary endpoint (expansion): antitumour activity based on ORR Secondary endpoints: PK parameters, ADA, tumoural baseline PD-L1, anti-tumour activity (OR, DoR, DCR, PFS, OS) 	<ul style="list-style-type: none"> FPCD: Q2 2018 Data anticipated: >2024



**Oncology:
early-stage development**



AZD0171 (anti-LIF mAb)

Cancer

Approved medicines

Late-stage development

Early development

Oncology

CVRM

R&I

Other

V&I

Rare Disease

Trial	Population	Patients	Design	Endpoints	Status
Phase II NCT04999969	1L metastatic pancreatic ductal adenocarcinoma	115	<ul style="list-style-type: none">Open-label, non-randomised trialAZD0171 + <i>Imfinzi</i> + gemcitabine, nab-paclitaxel	<ul style="list-style-type: none">Primary endpoints: safety, OS at 12 monthsSecondary endpoints: ORR, DoR and PFS	<ul style="list-style-type: none">FPCD: Q1 2022Data anticipated: H2 2024



AZD0486 (TNB-486, CD19/CD3 next-generation bispecific T-cell engager)

Haematologic malignancies

Trial	Population	Patients	Design	Endpoints	Status
Phase I NCT04594642	R/R B-cell non-Hodgkin lymphoma	116	<ul style="list-style-type: none"> Multi-centre, open-label, dose escalation and dose expansion trial AZD0486 	<ul style="list-style-type: none"> Primary endpoints: safety and tolerability, MTD and/or RP2D and PK parameters Secondary endpoints: clinical activity of AZD0486 monotherapy and ADA titers for AZD0486 monotherapy 	<ul style="list-style-type: none"> FPCD: Q1 2021 Data anticipated: H2 2023



AZD1390 (ATM inhibitor)

Cancer

Approved medicines

Late-stage development

Early development

Oncology

CVRM

R&I

Other

V&I

Rare Disease

Trial	Population	Patients	Design	Endpoints	Status
Phase I NCT03423628	Recurrent glioblastoma eligible for re-irradiation, brain metastases and leptomeningeal disease, newly-diagnosed glioblastoma patients	120	<ul style="list-style-type: none">Open-label trialArm 1: recurrent GBM, AZD1390 + RT in dose escalation cohortsArm 3: primary GBM, AZD1390 + RT in dose escalation cohorts	<ul style="list-style-type: none">Primary endpoints: safety, tolerability and MTDSecondary endpoints: PK parameters and preliminary assessment of anti-tumour activity	<ul style="list-style-type: none">FPCD: Q2 2018Data anticipated: H2 2024



saruparib (AZD5305, PARP1 inhibitor)

Solid tumours

Trial	Population	Patients	Design	Endpoints	Status
Phase I/IIa PETRA NCT04644068	Advanced solid tumours	604	<ul style="list-style-type: none"> Modular, open-label, multi-centre dose escalation and expansion trial Module 1: saruparib Module 2: saruparib + paclitaxel Module 3: saruparib + carboplatin +/- paclitaxel Module 4: saruparib + <i>Enhertu</i> Module 5: saruparib + datopotamab deruxtecan Module 6: saruparib + camizestrant 	<ul style="list-style-type: none"> Primary endpoints: safety and tolerability, PK parameters Secondary endpoint: efficacy 	<ul style="list-style-type: none"> FPCD: Q4 2020 Data anticipated: >2024
Phase I/IIa PETRANHA NCT05367440	Metastatic prostate cancer	172	<ul style="list-style-type: none"> Multi-arm, open-label, non-randomised, multi-centre trial of saruparib in combination with new hormonal agents in patients with metastatic prostate cancer Arm 1: saruparib + enzalutamide Arm 2: saruparib + abiraterone acetate Arm 3: saruparib + darolutamide 	<ul style="list-style-type: none"> Primary endpoints: safety and tolerability Secondary endpoints: PK parameters and efficacy 	<ul style="list-style-type: none"> FPCD: Q2 2022 Data anticipated: >2024
Phase I NCT05573724	Locally advanced, unresectable or metastatic solid tumours	16	<ul style="list-style-type: none"> Part A: to assess the effect of multiple doses of itraconazole on the single-dose PK parameters of saruparib which will last up to 13 days and follows a non-randomised, open-label, 2 intervention design Part B: option to continue with saruparib monotherapy after completing Part A and whilst obtaining clinical benefit 	<ul style="list-style-type: none"> Primary endpoint: PK parameters Secondary endpoints: safety and tolerability 	<ul style="list-style-type: none"> FPCD: Q4 2022 LPCD: Q2 2023 Data anticipated: H2 2023
Phase I ASCERTAIN NCT05938270	Newly diagnosed prostate cancer	120	<ul style="list-style-type: none"> Open-label, randomised, multi-centre trial 	<ul style="list-style-type: none"> Primary endpoint: to assess the effects of treatment on γH2AX change Secondary endpoints: safety and tolerability, impact on surgical feasibility and change in Ki67 	<ul style="list-style-type: none"> FPCD: Q3 2023 Data anticipated: >2024



AZD5335 (anti-FR α TOP1i ADC)

Solid tumours, ovarian cancer, lung cancer

Trial	Population	Patients	Design	Endpoints	Status
Phase I/II FONTANA NCT05797168	Advanced solid tumour malignancies	150	<ul style="list-style-type: none"> Module 1: AZD5335 monotherapy Module 2: AZD5335 in combination with saruparib 	<ul style="list-style-type: none"> Primary endpoints: safety and tolerability Secondary endpoints: efficacy and PK parameters 	<ul style="list-style-type: none"> Data anticipated: >2024



AZD5863 (CLDN18.2 x CD3 bispecific antibody)

Solid tumours

Trial	Population	Patients	Design	Endpoints	Status
Phase I NCT06005493	Advanced or metastatic solid tumours with CLDN18.2 expression	200	<ul style="list-style-type: none"> Part A: dose escalation phase to determine the safety, tolerability, RP2D, and/or MTD of AZD5863 Part B: dose expansion phase to further characterize the safety profile and evaluate anti-tumor activity of AZD5863 	<ul style="list-style-type: none"> Primary endpoint (Part A): safety and tolerability Primary endpoint (Part B): safety, tolerability and preliminary anti-tumour activity Secondary endpoints: preliminary anti-cancer activity, PK parameters and immunogenicity 	<ul style="list-style-type: none"> FPCD: Q4 2023 Data anticipated: >2024



AZD8205 (B7H4 ADC)

Solid tumours

Approved medicines

Late-stage development

Early development

Oncology

CVRM

R&I

Other

V&I

Rare Disease

Trial	Population	Patients	Design	Endpoints	Status
Phase I/II NCT05123482	Breast cancer, BTC, ovarian cancer, endometrial cancer	280	<ul style="list-style-type: none">Open-label, non-randomised dose escalation, and randomised/non-randomised dose expansion trial in monotherapyAZD8205	<ul style="list-style-type: none">Primary endpoints: AE, SAE, DLTs, changes in lab and preliminary efficacy parametersSecondary endpoints: ORR, DCR, DoR, PFS, OS, PK parameters and ADA	<ul style="list-style-type: none">FPCD: Q1 2022Data anticipated: >2024



AZD9574 (PARP1-selective BBB inhibitor)

Solid tumours

Trial	Population	Patients	Design	Endpoints	Status
Phase I/IIa CERTIS-1 NCT05417594	Advanced solid malignancies	255	<ul style="list-style-type: none"> Modular, open-label, multi-centre dose escalation and expansion trial Module 1: AZD9574 monotherapy Module 2: AZD9574 + temozolomide Module 3: [¹¹C]AZ14193391 + AZD9574 or [¹¹C]AZ14193391 + AZD9574 + temozolomide Module 4: AZD9574 + <i>Enhertu</i> Module 5: AZD9574 + datopotamab deruxtecan 	<ul style="list-style-type: none"> Primary endpoints: safety and tolerability of AZD9574 as monotherapy and in combination with anti-cancer agents, determination of PARP1 occupancy in brain by AZD9574 at examined doses and plasma concentration and evaluation of safety of radioligand [¹¹C]AZ14193391 Secondary endpoints: PK parameters and efficacy of AZD9574 as monotherapy and in combination with anti-cancer agents 	<ul style="list-style-type: none"> FPCD: Q3 2022 Data anticipated: >2024



AZD9592 (EGFR-cMET TOP1i ADC)

Lung cancer

Approved medicines

Late-stage development

Early development

Oncology

CVRM

R&I

Other

V&I

Rare Disease

Trial	Population	Patients	Design	Endpoints	Status
Phase I EGRET NCT05647122	Advanced solid tumours including NSCLC and HNSCC	108	<ul style="list-style-type: none"> Escalation phase, open-label, multi-centre trial AZD9592 AZD9592 + <i>Tagrisso</i> 	<ul style="list-style-type: none"> Primary endpoints (escalation): safety and tolerability Primary endpoints (expansion): safety and tolerability, anti-tumour activity Secondary endpoints (escalation): PK parameters, immunogenicity, anti-tumour activity Secondary endpoints (expansion): PK parameters and immunogenicity 	<ul style="list-style-type: none"> FPCD: Q1 2023 Data anticipated: >2024



IPH5201 (CD39 mAb)

Solid tumours

Trial	Population	Patients	Design	Endpoints	Status
Phase I NCT04261075 Partnered (Innate Pharma)	Advanced solid tumours	57	<ul style="list-style-type: none"> Open-label, dose escalation trial to determine MTD of IPH5201 as monotherapy, or in combination with <i>Imfinzi</i> +/- oleclumab Part 1: IPH5201 monotherapy dose escalation to MTD Part 2: IPH5201 + <i>Imfinzi</i> dose escalation to MTD Part 3: IPH5201 + <i>Imfinzi</i> + oleclumab dose escalation to MTD Route of administration: i.v. Global trial – US and EU 	<ul style="list-style-type: none"> Primary endpoints: AE, SAE and DLT Secondary endpoints: OR, DC, PK parameters and ADA 	<ul style="list-style-type: none"> FPCD: Q1 2020 LPCD: Q2 2022 Data readout: Q1 2023



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

Solid tumours

Trial	Population	Patients	Design	Endpoints	Status
Phase I EudraCT: 2021-006406-73	Adults with recurrent or metastatic NSCLC, melanoma, colorectal adenocarcinoma, HNSCC, bladder carcinoma, TNBC, cervical squamous cell carcinoma and adenocarcinoma or microsatellite instability-high/mismatch repair-deficient solid tumours	42	<ul style="list-style-type: none"> Open-label, single-arm, single-centre trial with dose escalation and dose expansion components NT-125 	<ul style="list-style-type: none"> Primary endpoints (Phase Ia): incidence of AEs defined as DLTs Primary endpoints (Phase Ib): ORR per RECIST v.1.1 Secondary endpoints (Phase Ia): percentage of pre-screened and enrolled subjects that receive treatment Secondary endpoints (Phase Ib): percentage change tumour size, best percentage change tumor size, DoR, clinical benefit rate, TTP, PFS and OS 	<ul style="list-style-type: none"> FPCD: Q2 2023 Data anticipated: >2024



NT-175 (TP53-armored TCR)

Multiple cancers

Trial	Population	Patients	Design	Endpoints	Status
Phase I NCT05877599	Unresectable, advanced, and/or metastatic solid tumours positive for HLA-A*02:01 and the TP53 R175H mutation	24	<ul style="list-style-type: none"> Open-label, single-arm, multi-centre trial with dose escalation 	<ul style="list-style-type: none"> Primary endpoint: incidence of DLTs, TEAEs and SAEs Secondary endpoints: ORR per RECIST v.1.1, BOR, DOR, CBR (CR, PR, SD), TTR, PFS and OS 	<ul style="list-style-type: none"> FPCD: Q3 2023 Data anticipated: >2024



oleclumab (CD73 mAb)

Solid tumours

Trial	Population	Patients	Design	Endpoints	Status
Phase Ib/II NCT03611556	Pancreatic 1L and 2L with prior gemcitabine-based chemotherapy	339	<ul style="list-style-type: none"> 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. + <i>Imfinzi</i> 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. + <i>Imfinzi</i> i.v. Global trial – US, Norway, Spain and Australia 	<ul style="list-style-type: none"> Primary endpoints: safety and anti-tumour activity Secondary endpoints: PFS, PK parameters, immunogenicity, safety and anti-tumour activity 	<ul style="list-style-type: none"> FPCD: Q2 2018 LPCD: Q3 2022 Data readout: Q1 2023



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

Solid tumours

Trial	Population	Patients	Design	Endpoints	Status
Phase IIb GEMINI-GC NCT05702229 Partnered (Compugen)	Gastric cancer	80	<ul style="list-style-type: none"> Open-label gastric platform study Sub-study 1: volrustomig combined with XELOX or volrustomig combined with FOLFOX Sub-study 2: rilvegostomig combined with XELOX or rilvegostomig combined with FOLFOX 	<ul style="list-style-type: none"> Primary endpoints: safety and efficacy (ORR and PFS6) Secondary endpoints: DoR, OS, PK, ADA and safety 	<ul style="list-style-type: none"> FPCD: Q1 2023 Data anticipated: >2024
Phase IIb GEMINI-HBP NCT05775159 Partnered (Compugen)	HCC, BTC	180	<ul style="list-style-type: none"> Open-label hepatobiliary platform study HCC sub-study: <ul style="list-style-type: none"> Cohort 1A: volrustomig monotherapy Cohort 1B: volrustomig combination with bevacizumab Cohort 1C: volrustomig combination with lenvatinib BTC sub-study: <ul style="list-style-type: none"> Cohort 2A: rilvegostomig combination with gemcitabine and cisplatin Cohort 2B: volrustomig combination with gemcitabine and cisplatin 	<ul style="list-style-type: none"> Primary endpoints (HCC sub-study): safety and efficacy (ORR) Primary endpoints (BTC sub-study): safety and efficacy (PFS6) Secondary endpoints: DoR, OS, PK and ADA 	<ul style="list-style-type: none"> FPCD: Q2 2023 Data anticipated: H2 2024
Phase I/II ARTEMIDE-01 NCT04995523 Partnered (Compugen)	NSCLC	192	<ul style="list-style-type: none"> Open-label, dose escalation and dose expansion trial Part A: dose escalation in CPI-experienced NSCLC patients with rilvegostomig i.v. monotherapy Part B: dose expansion in CPI-experienced NSCLC patients with rilvegostomig i.v. monotherapy Part C: dose expansion in CPI-naive NSCLC patients with rilvegostomig i.v. monotherapy Part D: randomised dose expansion in CPI-naive NSCLC patients with rilvegostomig i.v. monotherapy Global trial 	<ul style="list-style-type: none"> Primary endpoints (Part A): safety, RP2D and MTD Primary endpoints (Part B): safety and efficacy (ORR) Primary endpoints (Part C): safety and efficacy (ORR) Primary endpoints (Part D): safety and efficacy (ORR) Secondary endpoints: PK parameters, PD (receptor occupancy), efficacy (DCR, DoR, DRR, PFS) 	<ul style="list-style-type: none"> FPCD: Q4 2021 Data anticipated: H1 2024



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

Cancer

Approved medicines

Late-stage development

Early development

Oncology

CVRM

R&I

Other

V&I

Rare Disease

Trial	Population	Patients	Design	Endpoints	Status
Phase I/IIa NCT04931654	NSCLC, other tumours	192	<ul style="list-style-type: none"> Open-label, non-randomised dose escalation and dose expansion trial Part A: dose escalation in post-IO NSCLC patients with sabestomig i.v. monotherapy Part B: dose expansion in post-IO and IO-naïve NSCLC patients with sabestomig i.v. monotherapy Global trial 	<ul style="list-style-type: none"> Primary endpoints: AE, SAE, DLTs and ORR Secondary endpoints: ORR, DCR, DoR, PFS, OS, PK parameters, ADA and ctDNA 	<ul style="list-style-type: none"> FPCD: Q4 2021 Data anticipated: >2024
Phase I/II NCT05216835	R/R classical Hodgkin lymphoma	180	<ul style="list-style-type: none"> Cohort A: dose escalation where patients with anti-PD-1/PD-L1 exposed R/R cHL will receive sabestomig Cohort B1: dose expansion where patients with anti-PD-1/PD-L1 exposed R/R cHL will receive sabestomig once the recommended Phase II dose (RP2D) has been determined Cohort B2: dose expansion where patients with anti-PD-1/PD-L1 naïve R/R cHL will receive sabestomig once the RP2D has been determined 	<ul style="list-style-type: none"> Primary endpoints (Cohort A): AE and DLTs Primary endpoints (Cohort B1): AE and ORR Primary endpoints (Cohort B2): AE and CRR Secondary endpoints (Cohort A): CRR, ORR, DoR, DoCR, PFS, OS, ADA and PK parameters Secondary endpoints (Cohort B1 and B2): DoR, DoCR, PFS, OS, ADA and PK parameters 	<ul style="list-style-type: none"> FPCD: Q2 2022 Data anticipated: >2024



**BioPharmaceuticals:
approved medicines and
late-stage pipeline**



Andexxa (anti-factor Xa reversal)

Haematology

Approved medicines
Late-stage development
Early development

Trial	Population	Patients	Design	Endpoints	Status
Phase IV I8-513 (post-launch) NCT03661528	Acute intracranial haemorrhage	1200	<ul style="list-style-type: none"> Arm 1: <i>Andexxa</i> Arm 2: usual care Global trial 	<ul style="list-style-type: none"> Primary endpoint: proportion of patients with good or excellent haemostatic efficacy as rated by an independent adjudication committee Secondary endpoint: change from baseline in anti-factor Xa activity 	<ul style="list-style-type: none"> FPCD: Q2 2019 Data readout: Q2 2023 Primary endpoint met
Phase II 19-515 NCT04233073	Urgent surgery	10	<ul style="list-style-type: none"> Arm 1: <i>Andexxa</i> 	<ul style="list-style-type: none"> 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 	<ul style="list-style-type: none"> FPCD: Q2 2021 LPCD: Q1 2022 Data readout: Q4 2022

Oncology

CVRM

R&I

Other

V&I

Rare Disease



Farxiga (SGLT2 inhibitor)

Heart failure and chronic kidney disease

Trial	Population	Patients	Design	Endpoints	Status
Phase III DELIVER NCT03619213	CHF patients with HFpEF	6263	<ul style="list-style-type: none"> Arm 1: <i>Farxiga</i> 10mg QD Arm 2: placebo Global trial – 21 countries 	<ul style="list-style-type: none"> 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) 	<ul style="list-style-type: none"> FPCD: Q4 2018 LPCD: Q1 2022 Data readout: Q2 2022 Primary endpoint met
Phase III DAPA-MI NCT04564742	Myocardial infarction	6400	<ul style="list-style-type: none"> Arm 1: <i>Farxiga</i> 10mg QD Arm 2: placebo Global trial – 2 countries 	<ul style="list-style-type: none"> Primary endpoint: time to the first occurrence of any of the components of the composite (hospitalisation for HF or CV death) 	<ul style="list-style-type: none"> FPCD: Q4 2020 Data readout: Q4 2023 Primary endpoint met



Lokelma (sodium zirconium cyclosilicate)

Hyperkalaemia

Trial	Population	Patients	Design	Endpoints	Status
Phase III DIALIZE-Outcomes NCT04847232	Recurrent hyperkalaemia on chronic haemodialysis	2800	<ul style="list-style-type: none"> Arm 1: <i>Lokelma</i> 5g to 15g QD for 4 weeks on non-dialysis days; thereafter, adjusted monthly Arm 2: placebo QD Global trial – 26 countries 	<ul style="list-style-type: none"> Primary endpoint: time to first occurrence of SCD, stroke or hospitalisation, intervention or ED visit due to arrhythmia 	<ul style="list-style-type: none"> FPCD: Q3 2021 Data anticipated: >2024
Phase III STABILIZE-CKD NCT05056727	Patients with CKD and hyperkalaemia or at risk of hyperkalaemia	1360	<ul style="list-style-type: none"> Open-label <i>Lokelma</i> (10g TID or 5g QD) for up to 72 hours, followed by 3 months open-label treatment with <i>Lokelma</i> (5g QOD to 15g QD) and uptitration of lisinopril or valsartan; thereafter, patients are randomised to a 24 month treatment: <ul style="list-style-type: none"> Arm 1: <i>Lokelma</i> (5g QOD to 15g QD) and lisinopril or valsartan Arm 2: placebo and lisinopril or valsartan Global trial – 20 countries 	<ul style="list-style-type: none"> Primary endpoints: total slope (eGFR measurements starting at randomisation) and chronic slope (eGFR measurements starting at 12 weeks after randomisation) 	<ul style="list-style-type: none"> FPCD: Q4 2021 Data anticipated: >2024



roxadustat (HIF PH inhibitor)

Anaemia

Approved medicines

Late-stage development

Early development

Oncology

CVRM

R&I

Other

V&I

Rare Disease

Trial	Population	Patients	Design	Endpoints	Status
Phase III MATTERHORN NCT03263091 Partnered (FibroGen)	Anaemia in lower-risk MDS patients	184	<ul style="list-style-type: none">Open-label roxadustat lead-inArm 1: roxadustatArm 2: placeboGlobal trial	<ul style="list-style-type: none">Primary endpoint: proportion of patients achieving transfusion independence	<ul style="list-style-type: none">FPCD: Q3 2017Data readout: Q2 2023Primary endpoint not met
Phase II/III NCT03303066 Partnered (FibroGen)	Anaemia in lower-risk MDS patients	43	<ul style="list-style-type: none">Open-label roxadustat lead-inArm 1: roxadustatArm 2: placeboGlobal trial	<ul style="list-style-type: none">Primary endpoint: proportion of patients achieving transfusion independence	<ul style="list-style-type: none">FPCD: Q2 2018LPCD: Q1 2023Data readout: Q2 2023Primary endpoint met



baxdrostat (selective aldosterone synthase inhibitor)

Hypertension

Trial	Population	Patients	Design	Endpoints	Status
Phase II SPARK NCT04605549	Patients with primary aldosteronism	18	<ul style="list-style-type: none"> Arm 1: baxdrostat 2-8mg QD US only 	<ul style="list-style-type: none"> Primary endpoints: safety and tolerability in patients with PA at doses from 2 to 8mg per day for 12 weeks and the reduction in SBP patients with PA after 12 weeks Secondary endpoints: reduction in DBP as a function of dose in patients with PA after 12 weeks of treatment, change in serum potassium and requirement for potassium supplementation and change in serum sodium and requirement for fluid or mineral replacement 	<ul style="list-style-type: none"> FPCD: Q3 2022 Data anticipated: H2 2024
Phase II HALO-OLE NCT05459688	Patients with hypertension who have completed study CIN-107-124	175	<ul style="list-style-type: none"> Arm 1: baxdrostat 2mg QD US only 	<ul style="list-style-type: none"> Primary endpoints: safety and tolerability 	<ul style="list-style-type: none"> FPCD: Q2 2022 Data anticipated: H2 2023
Phase II FigHTN NCT05432167	Patients with uncontrolled hypertension and CKD	300	<ul style="list-style-type: none"> Arm 1: baxdrostat (low dose) Arm 2: baxdrostat (high dose) Arm 3: placebo US only 	<ul style="list-style-type: none"> Primary endpoint: change from baseline in mean seated systolic blood pressure vs. placebo at Week 26 Secondary endpoint: to evaluate the treatment effect on SBP at Week 26 by dosing strategy 	<ul style="list-style-type: none"> FPCD: Q2 2022 Data anticipated: H2 2024



eplontersen (ligand-conjugated antisense)

ATTR

Approved medicines

Late-stage development

Early development

Oncology

CVRM

R&I

Other

V&I

Rare Disease

Trial	Population	Patients	Design	Endpoints	Status
Phase III CARDIO-TTRansform NCT04136171 Partnered (Ionis Pharmaceuticals, Inc.)	Hereditary or wild-type transthyretin-mediated amyloid cardiomyopathy (ATTR-CM)	1438	<ul style="list-style-type: none"> Arm 1: eplontersen s.c. Arm 2: placebo 	<ul style="list-style-type: none"> Primary endpoints: composite outcome of CV mortality and recurrent CV clinical events at Week 140 Secondary endpoints: 6MWT, KCCQ, CV events and CV mortality 	<ul style="list-style-type: none"> FPCD: Q1 2020 Data anticipated: >2024
Phase III NEURO-TTRansform NCT04136184 Partnered (Ionis Pharmaceuticals, Inc.)	Hereditary transthyretin-mediated amyloid polyneuropathy (hATTR-PN)	168	<ul style="list-style-type: none"> Arm 1: eplontersen s.c. Arm 2: inotersen s.c. 	<ul style="list-style-type: none"> Primary endpoints (at Week 35): change from baseline in mNIS+7 and percent change from baseline in TTR concentration Secondary endpoint (Week 35): changes from baseline in Norfolk QOL Primary endpoints (at Week 66): change from baseline in mNIS+7, change from baseline in the Norfolk QoL-DN Questionnaire and percent change from baseline in TTR concentration 	<ul style="list-style-type: none"> FPCD: Q1 2020 LPCD: Q3 2023 Data readout: Q2 2022 Co-primary endpoints met at Week 35 and Week 66



mitiperstat (MPO inhibitor)

Cardiovascular disease

Approved medicines

Late-stage development

Early development

Oncology

CVRM

R&I

Other

V&I

Rare Disease

Trial	Population	Patients	Design	Endpoints	Status
Phase IIb ENDEAVOR NCT04986202	HFpEF	711	<ul style="list-style-type: none">• Randomised, double-blind• Arm 1: 2.5mg mitiperstat• Arm 2: 5mg mitiperstat• Arm 3: placebo• Global trial	<ul style="list-style-type: none">• Primary endpoints: safety and efficacy	<ul style="list-style-type: none">• FPCD: Q3 2021• Data anticipated: H1 2024



tozorakimab (IL-33 ligand mAb)

Diabetic kidney disease

Trial	Population	Patients	Design	Endpoints	Status
Phase II NCT04170543	Adult patients with diabetic kidney disease	581	<ul style="list-style-type: none"> • Arm 1: tozorakimab dose 1 + <i>Farxiga</i> • Arm 2: tozorakimab dose 2 + <i>Farxiga</i> • Arm 3: tozorakimab dose 3 + <i>Farxiga</i> • Arm 4: tozorakimab dose 4 + <i>Farxiga</i> • Arm 5: placebo + <i>Farxiga</i> • Global trial – US, Canada, Japan and additional countries 	<ul style="list-style-type: none"> • Primary endpoint: change from baseline in UACR compared to placebo at 24 weeks • Secondary endpoints: safety and other efficacy measures 	<ul style="list-style-type: none"> • FPCD: Q4 2019 • LPCD: Q3 2022 • Data anticipated: H2 2023



zibotentan/dapagliflozin (ETA receptor antagonist/SGLT2 inhibitor)

Chronic kidney disease

Trial	Population	Patients	Design	Endpoints	Status
Phase III ZENITH High Proteinuria NCT06087835	CKD and high proteinuria	1500	<ul style="list-style-type: none"> Randomised, parallel, multi-centre, double-blinded trial Arm 1: zibotentan/dapagliflozin dose A or dose B Arm 2: dapagliflozin Global trial 	<ul style="list-style-type: none"> Primary endpoint: change in eGFR from baseline Secondary endpoints: change in UPCR from baseline to each participant's mean level; change in UACR from baseline to each participant's mean level; time to the first occurrence of any of the components of the renal composite endpoint of 40% sustained decline in eGFR or ESKD or renal death 	<ul style="list-style-type: none"> Initiating Data anticipated: >2024
Phase IIb ZENITH-CKD NCT04724837	CKD	447	<ul style="list-style-type: none"> Arm 1: zibotentan dose A + <i>Farxiga</i> 10mg QD Arm 2: zibotentan dose B + <i>Farxiga</i> 10mg QD Arm 3: <i>Farxiga</i> 10mg + placebo QD Global trial 	<ul style="list-style-type: none"> Primary endpoint: change in log-transformed UACR from baseline to Week 12 zibotentan dose B/<i>Farxiga</i> 10mg vs. <i>Farxiga</i> 10mg Secondary endpoints: change in log-transformed UACR from baseline to Week 12 zibotentan dose A/<i>Farxiga</i> 10mg vs. <i>Farxiga</i> 10mg; change in blood pressure, least squares mean change of UACR, change in eGFR at predetermined timepoints and number of participants experiencing adverse events 	<ul style="list-style-type: none"> FPCD: Q2 2021 Data readout: Q3 2023 Primary endpoint met



zibotentan/dapagliflozin (ETA receptor antagonist/SGLT2 inhibitor)

Liver cirrhosis

Trial	Population	Patients	Design	Endpoints	Status
Phase II ZEAL NCT05516498	Part A: participants with Child-Pugh A cirrhosis with features of portal hypertension and with no history of decompensation events Part B: participants with a broader range of Child-Pugh A and Child-Pugh B cirrhosis with more severe disease	140	<ul style="list-style-type: none"> Phase IIa/b multi-centre, randomised, double-blind, placebo-controlled, parallel group dose-ranging trial Part A Arm 1: placebo Part A Arm 2: zibotentan dose B + <i>Farxiga</i> Part B Arm 1: placebo Part B Arm 2: placebo + <i>Farxiga</i> Part B Arm 3: zibotentan dose A + <i>Farxiga</i> Part B Arm 4: zibotentan dose B + <i>Farxiga</i> Part B Arm 5: zibotentan dose C + <i>Farxiga</i> Global trial 	<ul style="list-style-type: none"> Primary endpoint (Part A): absolute change in HVPG from baseline to Week 6 comparing zibotentan and <i>Farxiga</i> in combination vs. placebo Primary endpoint (Part B): HVPG response from baseline to Week 6 comparing zibotentan and <i>Farxiga</i> in combination and <i>Farxiga</i> monotherapy vs. placebo 	<ul style="list-style-type: none"> FPCD: Q4 2022 Data anticipated: H2 2024



Airsupra (PT027, SABA/ICS, pMDI)

Asthma

Trial	Population	Patients	Design	Endpoints	Status
Phase III MANDALA NCT03769090 Managed by Avillion (Avillion)	Moderate to severe asthma	3132	<ul style="list-style-type: none"> Randomised, double-blind, multi-centre, parallel group Treatments: minimum 24-week treatment period BDA (budesonide albuterol) MDI 80/180µg prn BDA MDI 160/180µg prn AS (albuterol sulphate) MDI 180µg prn Global trial 	<ul style="list-style-type: none"> 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 	<ul style="list-style-type: none"> FPCD: Q4 2018 LPCD: Q1 2021 Data readout: Q3 2021 Primary endpoint met
Phase III DENALI NCT03847896 Managed by Avillion (Avillion)	Mild to moderate asthma	1001	<ul style="list-style-type: none"> Randomised, double-blind, multi-centre and parallel-group Treatments: 12-week treatment period BDA MDI 80/180µg QID BDA MDI 160/180µg QID BD MDI 160µg QID AS MDI 180µg QID placebo MDI QID Global trial 	<ul style="list-style-type: none"> Dual primary endpoints: change from baseline in FEV1 AUC0-6 hours over 12 weeks; change from baseline in trough FEV1 at Week 12 	<ul style="list-style-type: none"> FPCD: Q2 2019 LPCD: Q2 2021 Data readout: Q3 2021 Dual primary endpoints met
Phase III TYREE NCT04234464 Managed by Avillion (Avillion)	Asthma with exercise induced bronchoconstriction	60	<ul style="list-style-type: none"> Randomised, double-blind, multi-centre crossover Treatments: single-dose BDA MDI 160/180µg placebo MDI QID US only 	<ul style="list-style-type: none"> Primary endpoint: maximum percentage fall from post-dose, pre-exercise baseline in FEV1 observed up to 60 minutes post-exercise challenge 	<ul style="list-style-type: none"> FPCD: Q1 2020 LPCD: Q3 2020 Data readout: Q4 2020 Primary endpoint met



Breztri/ Trixeo (LAMA/LABA/ICS)

Asthma

Trial	Population	Patients	Design	Endpoints	Status
Phase III KALOS NCT04609878	Severe asthma	2200	<ul style="list-style-type: none"> Randomised, double-blind, double-dummy, parallel group and multi-centre trial Treatments (24- to 52-week variable length) BGF 320/28.8/9.6µg BID MDI BGF 320/14.4/9.6µg BID MDI BFF 320/9.6µg BID MDI Symbicort 320/9µg BID pMDI Global trial 	<ul style="list-style-type: none"> Primary endpoint: change from baseline in FEV1 AUC0-3 at Week 24 Secondary endpoint: change from baseline in morning pre-dose trough FEV1 at Week 24 	<ul style="list-style-type: none"> FPCD: Q1 2021 Data anticipated: >2024
Phase III LOGOS NCT04609904	Severe asthma	2200	<ul style="list-style-type: none"> Randomised, double-blind, double dummy, parallel group and multi-centre trial Treatments (24- to 52-week variable length) BGF 320/28.8/9.6µg BID MDI BGF 320/14.4/9.6µg BID MDI BFF 320/9.6µg BID MDI Symbicort 320/9µg BID pMDI Global trial 	<ul style="list-style-type: none"> Primary endpoint: change from baseline in FEV1 AUC0-3 at Week 24 Secondary endpoint: change from baseline in morning pre-dose trough FEV1 at Week 24 	<ul style="list-style-type: none"> FPCD: Q1 2021 Data anticipated: >2024
Phase III VATHOS NCT05202262	Moderate asthma	630	<ul style="list-style-type: none"> Randomised, double-blind, parallel group, multi-centre trial Treatments (24-week) BFF 320/9.6µg BID MDI BFF 160/9.6µg BID MDI BD 320µg BID MDI Open-label Symbicort TBH 320/9µg BID Global trial 	<ul style="list-style-type: none"> Primary endpoint: change from baseline in FEV1 AUC0-3 at Week 24 	<ul style="list-style-type: none"> FPCD: Q1 2022 Data anticipated: H2 2024
Phase III LITHOS NCT05755906	Mild to moderate asthma	340	<ul style="list-style-type: none"> Randomised, double-blind, parallel group and multi-centre Treatments (12-week) BFF 160/9.6µg BID MDI BD 160µg BID MDI Global trial 	<ul style="list-style-type: none"> Primary endpoint: Change from baseline in forced expiratory volume in 1 second (FEV1) area under the curve 0 to 3 hours (AUC0-3) at Week 12 	<ul style="list-style-type: none"> FPCD: Q1 2023 Data anticipated: H2 2024



Breztri/ Trixeo (LAMA/LABA/ICS)

COPD

Trial	Population	Patients	Design	Endpoints	Status
Phase III NCT05573464	Moderate to very severe COPD	542	<ul style="list-style-type: none"> Randomised, double-blind, 12-week (with an extension to 52 weeks in a subset of participants), parallel-group, multi-centre trial BGF MDI HFO 160/7.2/4.8µg (2 inhalations BID) BGF MDI HFA 160/7.2/4.8µg (2 inhalations BID) 	<ul style="list-style-type: none"> Primary endpoints: number of participants with AEs/SAEs and potentially clinically significant changes in Digital 12-lead Holter ECG, laboratory values, blood pressure, pulse rate, respiratory rate and body temperature 	<ul style="list-style-type: none"> FPCD: Q3 2022 Data anticipated: H2 2024
Phase III ATHLOS NCT06067828	COPD	180	<ul style="list-style-type: none"> Randomised, double-blind, three-treatment, three-period, cross-over trial Treatments (2-week treatment periods, 2-week washout between treatments) Arm 1: BGF 320/14.4/9.6µg BID MDI Arm 2: BFF 320/9.6µg BID MDI Arm 3: placebo BID MDI 	<ul style="list-style-type: none"> Primary endpoint: change from baseline in isotime IC Secondary endpoint: change from baseline in constant work rate cycle ergometry endurance time 	<ul style="list-style-type: none"> FPCD: Q4 2023 Data anticipated: >2024
Phase I NCT05477108	Healthy volunteers	108	<ul style="list-style-type: none"> Randomised, double-blind, single-dose, single-centre, partial-replicate, 3-way crossover trial BGF MDI HFO 160/7.2/4.8µg (single dose of 4 inhalations) BGF MDI HFA 160/7.2/4.8µg (single dose of 4 inhalations) 	<ul style="list-style-type: none"> Primary endpoint: AUCinf, AUClast and Cmax 	<ul style="list-style-type: none"> FPCD: Q3 2022 Data anticipated: H2 2023
Phase I NCT05569421	Healthy volunteers	108	<ul style="list-style-type: none"> Randomised, double-blind, single-dose, single-centre, partial-replicate, 3-way crossover trial BGF MDI HFO 160/7.2/4.8µg (single dose of 4 inhalations) BGF MDI HFA 160/7.2/4.8µg (single dose of 4 inhalations) 	<ul style="list-style-type: none"> Primary endpoint: AUCinf, AUClast and Cmax 	<ul style="list-style-type: none"> FPCD: Q4 2022 Data anticipated: H1 2024



Fasenra (IL-5R mAb)

Nasal polyposis and other eosinophilic diseases

Trial	Population	Patients	Design	Endpoints	Status
Phase III OSTRO NCT03401229	Patients with severe bilateral nasal polyps who are still symptomatic despite SoC therapy; age 18 to 75 years	413	<ul style="list-style-type: none"> Arm 1: <i>Fasenra</i> 30mg Q8W s.c. Arm 2: placebo s.c. 56-week trial Global trial – 8 countries 	<ul style="list-style-type: none"> Primary endpoint: effect of <i>Fasenra</i> on nasal polyp burden and on patient reported nasal blockage 	<ul style="list-style-type: none"> FPCD: Q1 2018 LPCD: Q2 2019 Data readout: Q3 2020 Co-primary endpoints met
Phase III ORCHID NCT04157335	Patients with eosinophilic chronic rhinosinusitis with severe nasal polyposis; age 18 to 75 years	276	<ul style="list-style-type: none"> Arm 1: <i>Fasenra</i> 30mg Q8W s.c. Arm 2: placebo Q8W s.c. 56-week trial Global trial – 10 countries 	<ul style="list-style-type: none"> Primary endpoints: change in endoscopic total nasal polyp score and change in mean nasal blockage score 	<ul style="list-style-type: none"> FPCD: Q4 2019 Data anticipated: H2 2024
Phase III MANDARA NCT04157348	Patients with R/R EGPA on corticosteroid therapy with or without stable immunosuppressive therapy; age 18 years and older	140	<ul style="list-style-type: none"> Arm 1: <i>Fasenra</i> 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 	<ul style="list-style-type: none"> Primary endpoint: proportion of patients achieving remission (BVAS=0 and OCS dose \leq4mg/day) at Week 36 and Week 48 	<ul style="list-style-type: none"> FPCD: Q4 2019 Data readout: Q3 2023 Primary endpoint met
Phase III NATRON NCT04191304	Patients with HES (history of persistent eosinophilia >1500 cells/ μ L 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	<ul style="list-style-type: none"> Arm 1: <i>Fasenra</i> 30mg Q4W s.c. Arm 2: placebo Q4W s.c. 24-week trial with a minimum 1-year open label extension Global trial – 9 to 12 countries 	<ul style="list-style-type: none"> Primary endpoint: time to first HES worsening/flare 	<ul style="list-style-type: none"> FPCD: Q3 2020 Data anticipated: H1 2024



Fasenra (IL-5R mAb)

Severe, uncontrolled asthma and COPD

Trial	Population	Patients	Design	Endpoints	Status
Phase III MIRACLE NCT03186209	Severe, uncontrolled asthma despite background controller medication, MD and HD ICS + LABA ± chronic OCS; age 12 to 75 years	695	<ul style="list-style-type: none"> Arm 1: <i>Fasenra</i> 30mg Q8W s.c. Arm 2: placebo s.c. 56-week trial 	<ul style="list-style-type: none"> Primary endpoint: annual asthma exacerbation rate Secondary endpoints: pulmonary function, asthma symptoms and other asthma control metrics 	<ul style="list-style-type: none"> FPCD: Q4 2017 LPCD: Q4 2021 Data readout: Q1 2023 Primary endpoint met
Phase III RESOLUTE NCT04053634	Patients with moderate to very severe COPD with a history of frequent exacerbations on a background triple therapy (ICS/LABA/LAMA); age 40 to 85 years	642	<ul style="list-style-type: none"> Double-blind, placebo-controlled trial Arm 1: <i>Fasenra</i> 100mg Q8W s.c. Arm 2: placebo Q8W s.c. 56-week treatment Global trial – 26 countries 	<ul style="list-style-type: none"> Primary endpoint: annualised rate of moderate or severe exacerbations over 56 weeks 	<ul style="list-style-type: none"> FPCD: Q4 2019 Data anticipated: >2024



Saphnelo (type I interferon receptor mAb)

Lupus (SLE/LN)

Approved medicines
Late-stage development
Early development

Trial	Population	Patients	Design	Endpoints	Status
Phase III TULIP-SC NCT04877691 Partnered (BMS)	Moderate to severe SLE	360	<ul style="list-style-type: none"> Arm 1: <i>Saphnelo</i> s.c. Arm 2: placebo s.c. Global trial 	<ul style="list-style-type: none"> Primary endpoint: BICLA at Week 52 	<ul style="list-style-type: none"> FPCD: Q3 2021 Data anticipated: >2024
Phase III AZALEA-SLE NCT04931563 Partnered (BMS)	Moderate to severe SLE patients	260	<ul style="list-style-type: none"> Arm 1: 300mg <i>Saphnelo</i> i.v. Q4W Arm 2: placebo i.v. Q4W Asia only 	<ul style="list-style-type: none"> Primary endpoint: BICLA at Week 52 	<ul style="list-style-type: none"> FPCD: Q4 2021 Data anticipated: >2024
Phase III IRIS NCT05138133 Partnered (BMS)	Active, proliferative LN	360	<ul style="list-style-type: none"> Arm 1: <i>Saphnelo</i> i.v. Arm 2: placebo i.v. 	<ul style="list-style-type: none"> Primary endpoint: CRR at Week 52 	<ul style="list-style-type: none"> FPCD: Q2 2022 Data anticipated: >2024

Oncology
CVRM
R&I
Other
V&I
Rare Disease



Saphnelo (type I interferon receptor mAb)

Sclerosis

Approved medicines
Late-stage development
Early development

Trial	Population	Patients	Design	Endpoints	Status
Phase III DAISY NCT05925803 Partnered (BMS)	Systemic sclerosis	306	<ul style="list-style-type: none">Arm 1: <i>Saphnelo</i> s.c.Arm 2: placebo s.c.	<ul style="list-style-type: none">Primary endpoint: CRIS-25 at Week 52	<ul style="list-style-type: none">InitiatingData anticipated >2024

Oncology
CVRM
R&I
Other
V&I
Rare Disease



Tezspire (TSLP mAb)

CRSwNP, COPD and EoE

Trial	Population	Patients	Design	Endpoints	Status
Phase III WAYPOINT NCT04851964 Partnered (AMGEN)	Severe chronic rhinosinusitis with nasal polyps; age 18 years and older	400	<ul style="list-style-type: none"> Arm 1: <i>Tezspire</i> s.c. Arm 2: placebo s.c. 52-week trial Global trial – 10 countries 	<ul style="list-style-type: none"> Co-primary endpoint: nasal polyp score and participant reported nasal congestion 	<ul style="list-style-type: none"> FPCD: Q2 2021 LPCD: Q4 2023 Data anticipated: H2 2024
Phase III CROSSING NCT05583227 Partnered (AMGEN)	Adult and paediatric aged 12 years and older with eosinophilic esophagitis	360	<ul style="list-style-type: none"> Arm 1: <i>Tezspire</i> s.c. low dose Arm 2: <i>Tezspire</i> s.c. high dose Arm 3: placebo 52-week trial Global trial – 20+ countries 	<ul style="list-style-type: none"> Co-primary endpoints: histologic response of peak esophageal eosinophil per HPF count of ≤ 6 across all available esophageal levels and change from baseline in Dysphagia Symptom Questionnaire score 	<ul style="list-style-type: none"> FPCD: Q1 2023 Data anticipated: >2024
Phase IIa COURSE NCT04039113 Partnered (AMGEN)	Moderate to very severe COPD; age 40 to 80	338	<ul style="list-style-type: none"> Arm 1: <i>Tezspire</i> s.c. Arm 2: placebo s.c. 52-week trial Global trial – 10 countries 	<ul style="list-style-type: none"> Primary endpoint: rate of moderate or severe COPD exacerbations 	<ul style="list-style-type: none"> FPCD: Q3 2019 LPCD: Q4 2022 Data anticipated: H1 2024



Tezspire (TSLP mAb)

Severe, uncontrolled asthma

Trial	Population	Patients	Design	Endpoints	Status
Phase III NAVIGATOR NCT03347279 Partnered (AMGEN)	Severe asthma; age 12 to 80 years	1061	<ul style="list-style-type: none"> Arm 1: <i>Tezspire</i> s.c. Arm 2: placebo s.c. 52-week trial Global trial – 18 countries 	<ul style="list-style-type: none"> Primary endpoint: annual asthma exacerbation rate Secondary endpoints: change from baseline in pre-BD FEV1, asthma related QoL (AQLQ(S)+12) and asthma control (ACQ-6) 	<ul style="list-style-type: none"> FPCD: Q1 2018 LPCD: Q3 2019 Data readout: Q4 2020 Primary endpoint met
Phase III DIRECTION NCT03927157 Partnered (AMGEN)	Severe asthma; age 18 to 80 years	405	<ul style="list-style-type: none"> Arm 1: <i>Tezspire</i> s.c. Arm 2: placebo s.c. 52-week trial Regional trial (Asia) – 3 countries 	<ul style="list-style-type: none"> Primary endpoint: annual asthma exacerbation rate Secondary endpoints: change from baseline in pre-BD FEV1, asthma related QoL (AQLQ(S)+12) and asthma control (ACQ-6) 	<ul style="list-style-type: none"> FPCD: Q3 2019 LPCD: Q2 2023 Data anticipated: H2 2024
Phase III SUNRISE NCT05398263 Partnered (AMGEN)	Severe asthma; age 18 to 80 years	207	<ul style="list-style-type: none"> Arm 1: <i>Tezspire</i> s.c. Arm 2: placebo s.c. 28-week trial Global trial – 15 countries 	<ul style="list-style-type: none"> Primary endpoint: categorised percent reduction from baseline in the daily maintenance OCS dose at Week 28 whilst maintaining asthma control 	<ul style="list-style-type: none"> FPCD: Q3 2022 Data anticipated: >2024



HFO1234ze (next-generation propellant)

pDMI

Approved medicines

Late-stage development

Early development

Oncology

CVRM

R&I

Other

V&I

Rare Disease

Trial	Population	Patients	Design	Endpoints	Status
Phase III NCT05755932	Muciliary clearance in healthy volunteers	30	<ul style="list-style-type: none"> Randomised, double-blind, multi-site, two-way crossover trial with propellant only Arm 1: HFO MDI; 6 inhalations BID for 7 days Arm 2: HFA MDI; 6 inhalations BID for 7 days 	<ul style="list-style-type: none"> Primary endpoint: change from baseline in MCC through 60 minutes following inhalation of 99m technetium sulfur colloid and gamma camera imaging Secondary endpoint: change from baseline in MCC at 3 hours following inhalation of 99m technetium sulfur colloid and gamma camera imaging 	<ul style="list-style-type: none"> FPCD: Q2 2023 Data anticipated: H2 2024
Phase III NCT05850494	Well-controlled or partially-controlled asthma	52	<ul style="list-style-type: none"> Randomised, multi-centre double-blind, single-dose crossover trial Arm 1: HFO propellant only MDI; 4 inhalations per dose Arm 2: HFA propellant only MDI; 4 inhalations per dose 	<ul style="list-style-type: none"> Primary endpoints: change from baseline FEV1 0 to 15 minutes post-dose, cumulative incidence of bronchospasm events and safety and tolerability 	<ul style="list-style-type: none"> FPCD: Q2 2023 Data anticipated: H1 2024



tozorakimab (IL-33 ligand mAb)

Severe viral LRTD

Approved medicines

Late-stage development

Early development

Oncology

CVRM

R&I

Other

V&I

Rare Disease

Trial	Population	Patients	Design	Endpoints	Status
Phase III TILIA NCT05624450	Adults hospitalised for viral lung infection requiring supplemental oxygen	2902	<ul style="list-style-type: none">• Randomised, double-blind, placebo-controlled, parallel group• Arm 1: tozorakimab dose i.v. + SoC• Arm 2: placebo i.v. + SoC• Global trial – 36 countries	<ul style="list-style-type: none">• Primary endpoint: progression to death or to invasive mechanical ventilation/extracorporeal membrane oxygenation• Secondary endpoints: safety and other efficacy measures	<ul style="list-style-type: none">• FPCD: Q4 2022• Data anticipated: >2024



tozorakimab (IL-33 ligand mAb)

Asthma

Trial	Population	Patients	Design	Endpoints	Status
Phase II FRONTIER-3 NCT04570657	Adults with uncontrolled moderate to severe asthma	250	<ul style="list-style-type: none"> Randomised, double-blind, placebo-controlled trial Arm 1: tozorakimab dose 1 s.c. Arm 2: tozorakimab dose 2 s.c. Arm 3: placebo s.c. Global trial – US, Argentina, Germany, Hungary, Poland, South Africa and UK 	<ul style="list-style-type: none"> Primary endpoint: change from baseline at Week 16 in FEV1 Secondary endpoints: safety and other efficacy measures 	<ul style="list-style-type: none"> FPCD: Q4 2020 LPCD: Q3 2022 Data readout: Q2 2023
Phase I NCT05070312	Healthy volunteers	36	<ul style="list-style-type: none"> Randomised, double-blind, placebo-controlled, dose ascending 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 only 	<ul style="list-style-type: none"> Primary endpoint: PK parameters Secondary endpoint: immunogenicity 	<ul style="list-style-type: none"> FPCD: Q3 2021 LPCD: Q4 2021 Data readout: Q2 2022



tozorakimab (IL-33 ligand mAb)

COPD

Trial	Population	Patients	Design	Endpoints	Status
Phase III OBERON NCT05166889	Adults with symptomatic COPD with a history of exacerbations	1060	<ul style="list-style-type: none"> Randomised, double-blind, placebo-controlled, parallel-group Treatment: 52-week Arm 1: tozorakimab dose 1 s.c. + SoC Arm 2: tozorakimab dose 2 s.c. + SoC Arm 3: placebo s.c. + SoC Global trial – 20 countries 	<ul style="list-style-type: none"> Primary endpoint: annualised rate of moderate to severe COPD exacerbations (former smokers) Secondary endpoints: annualised rate of moderate to severe COPD exacerbations (former or current smokers) and change in pre-BD FEV1, E-RS:COPD and SGRQ 	<ul style="list-style-type: none"> FPCD: Q1 2022 Data anticipated: >2024
Phase III TITANIA NCT05158387	Adults with symptomatic COPD with a history of exacerbations	1060	<ul style="list-style-type: none"> Randomised, double-blind, placebo-controlled, parallel-group Treatment: 52-week Arm 1: tozorakimab dose 1 s.c. + SoC Arm 2: tozorakimab dose 2 s.c. + SoC Arm 3: placebo s.c. + SoC Global trial – 19 countries 	<ul style="list-style-type: none"> Primary endpoint: annualised rate of moderate to severe COPD exacerbations (former smokers) Secondary endpoints: annualised rate of moderate to severe COPD exacerbations (former or current smokers) and change in pre-BD FEV1, E-RS:COPD and SGRQ 	<ul style="list-style-type: none"> FPCD: Q1 2022 Data anticipated: >2024
Phase III PROSPERO NCT05742802	Subjects who completed either OBERON or TITANIA will be offered the opportunity to consent (adults with symptomatic COPD with a history of exacerbations)	2544	<ul style="list-style-type: none"> Randomised, double-blind, placebo-controlled, parallel-group, long-term extension trial Treatment: 52-weeks Arm 1: tozorakimab dose 1 s.c. + SoC Arm 2: tozorakimab dose 2 s.c. + SoC Arm 3: placebo s.c. + SoC Global trial – 38 countries 	<ul style="list-style-type: none"> Primary endpoint: time to first severe COPD exacerbation in primary population of former smokers over the treatment period incorporating both the predecessor studies and PROSPERO Secondary endpoint: time to first severe COPD exacerbation in the overall population of current and former smokers 	<ul style="list-style-type: none"> FPCD: Q1 2023 Data anticipated: >2024



tozorakimab (IL-33 ligand mAb)

COPD

Trial	Population	Patients	Design	Endpoints	Status
Phase III MIRANDA NCT06040086	Adults with symptomatic COPD with a history of exacerbations	1240	<ul style="list-style-type: none"> Randomised, double-blind, placebo-controlled, parallel group Arm 1: tozorakimab dose s.c. + SoC Arm 2: placebo s.c. + SoC Global trial – 27 countries 	<ul style="list-style-type: none"> Primary endpoint: annualised rate of moderate to severe COPD exacerbations (former smokers) Secondary endpoints: annualised rate of moderate to severe COPD exacerbations (former or current smokers), annualised rate of severe COPD exacerbations (former and former or current smokers) and change in pre-BD FEV1, E-RS:COPD and SGRQ 	<ul style="list-style-type: none"> FPCD: Q4 2023 Data anticipated: >2024
Phase II NCT04631016	Adults with COPD and chronic bronchitis	137	<ul style="list-style-type: none"> Randomised, double-blind, placebo-controlled, parallel-group, PoC trial Arm 1: tozorakimab s.c. Arm 2: placebo s.c. Global trial – 15 countries 	<ul style="list-style-type: none"> Primary endpoint: change from baseline at Week 12 in FEV1 Secondary endpoints: safety and other efficacy measures 	<ul style="list-style-type: none"> FPCD: Q1 2021 Data readout: Q3 2023



Beyfortus (nirsevimab, RSV mAb-YTE)

Infection

Trial	Population	Patients	Design	Endpoints	Status
Phase III MELODY NCT03979313	Healthy infants (born 35 weeks 0 days or greater gestational age)	3012	<ul style="list-style-type: none"> Randomised, double-blind, placebo-controlled Arm 1: Beyfortus i.m. Arm 2: placebo i.m. Global trial – 31 countries 	<ul style="list-style-type: none"> Primary endpoint: efficacy Secondary endpoints: safety, PK parameters and ADA 	<ul style="list-style-type: none"> Data readout: Q3 2022 FPCD: Q2 2021 (safety cohort) LPCD: Q4 2021 (safety cohort) Data readout: Q3 2022 (safety cohort) Primary endpoint met FPCD: Q3 2019 (efficacy cohort) LPCD: Q1 2020 (efficacy cohort) Data readout: Q2 2021 (efficacy cohort) Primary endpoint met
Phase III CHIMES NCT05110261	Healthy infants (born 29 weeks 0 days or greater gestational age)	800	<ul style="list-style-type: none"> Randomised, double-blind, placebo-controlled Arm 1: Beyfortus i.m. Arm 2: placebo i.m. China only 	<ul style="list-style-type: none"> Primary endpoints efficacy Secondary endpoints: safety, PK parameters and ADA 	<ul style="list-style-type: none"> FPCD: Q4 2021 Data anticipated: >2024
Phase IIb NCT02878330	29- to 35-week gestational-age infants	1453	<ul style="list-style-type: none"> Randomised, double-blind, placebo-controlled trial Arm 1: Beyfortus i.m. Arm 2: placebo i.m. 	<ul style="list-style-type: none"> Primary endpoints: safety and efficacy 	<ul style="list-style-type: none"> FPCD: Q4 2016 LPCD: Q4 2017 Data readout: Q4 2018 Primary endpoint met
Phase II/III MEDLEY NCT03959488	High-risk pre-term (born 35 weeks 0 day or less gestational-age) CHD and CLD infants eligible to receive Synagis	925	<ul style="list-style-type: none"> Randomised, double-blind, palivizumab-controlled Arm 1: Beyfortus i.m. Arm 2: Synagis i.m. Global trial – 32 countries 	<ul style="list-style-type: none"> Primary endpoints: safety and tolerability Secondary endpoints: PK parameters, ADA and descriptive efficacy 	<ul style="list-style-type: none"> FPCD: Q3 2019 LPCD: Q4 2020 Data readout: Q2 2021 Safety objective met
Phase II MUSIC NCT04484935	Immunocompromised children who are ≤24 months of age at the time of dose administration	100	<ul style="list-style-type: none"> Open-label, uncontrolled, single-dose trial Beyfortus i.m. Route of administration: i.m. Global trial – 8 countries 	<ul style="list-style-type: none"> Primary endpoints: safety and tolerability Secondary endpoints: PK parameters, ADA and efficacy 	<ul style="list-style-type: none"> FPCD: Q3 2020 LPCD: Q1 2022 Data readout: Q2 2023 Primary endpoint met
Phase I NCT04840849	Healthy Chinese adults; age 18 to 45 years	24	<ul style="list-style-type: none"> Randomised, double-blind, placebo-controlled Arm 1: Beyfortus i.m. Arm 2: placebo i.m. China only 	<ul style="list-style-type: none"> Primary endpoint: PK parameters Secondary endpoints: ADA and safety 	<ul style="list-style-type: none"> FPCD: Q2 2021 LPCD: Q2 2021 Data readout: Q2 2022



Evusheld (AZD7442, tixagevimab + cilgavimab)

Prevention and treatment of COVID-19

Trial	Population	Patients	Design	Endpoints	Status
Phase II ENDURE NCT05375760	Adults and pediatric individuals (≥12 years of age weighing at least 40kg) who are moderate to severely immunocompromised due to an underlying disease or are taking immunosuppressive medications and therefore unable to mount an adequate immune response	251	<ul style="list-style-type: none"> Randomised, open-label, dose-ranging to assess safety, immunogenicity, PK and PD profiles in pre-exposure prophylaxis Arm 1: <i>Evusheld</i>, dose regimen 1 Arm 2: <i>Evusheld</i>, dose regimen 2 US only 	<ul style="list-style-type: none"> Primary endpoints: safety and tolerability, incidence of ADA Secondary endpoints: individual serum concentration; GMTs and GMFR in severe acute respiratory CoV-2 neutralizing antibodies 	<ul style="list-style-type: none"> FPCD: Q2 2022 LPCD: Q3 2022 Data anticipated: H1 2024
Phase I NCT05166421	Healthy adults; age ≥18 years	207	<ul style="list-style-type: none"> Open-label, randomised, three-arm, single-dose trial Arm 1: <i>Evusheld</i> administered as a single co-formulated dose (clonal cell line material) Arm 2: <i>Evusheld</i> administered as two separate doses (clonal cell line material) Arm 3: <i>Evusheld</i> administered as two separate doses (cell pool material) <i>Evusheld</i> (1:1:1) US only 	<ul style="list-style-type: none"> Primary endpoints: safety and PK parameters 	<ul style="list-style-type: none"> FPCD: Q1 2022 LPCD: Q3 2022 Data anticipated: H2 2023
Phase I TRUST NCT05281601 (No partner)	Pediatric participants ≥29 weeks gestational age to <18 years at increased risk of developing severe SARS-CoV-2 infection	100	<ul style="list-style-type: none"> Open-label, single-dose, three cohort trial Cohort 1: pre-exposure prophylaxis Cohort 2: mild-to-moderate COVID-19 Cohort 3: severe COVID-19 <i>Evusheld</i> US only 	<ul style="list-style-type: none"> Primary endpoints: safety, tolerability and PK parameters 	<ul style="list-style-type: none"> FPCD: Q1 2022 Data anticipated: >2024



AZD3152 (SARS-CoV-2 LAAB)

Prevention of COVID-19

Trial	Population	Patients	Design	Endpoints	Status
Phase III SUPERNOVA NCT05648110	Phase I: healthy adults; age 18 to 55 years Phase II: immunocompetent or immunoimpaired adults Phase III: 12 years of age or older with conditions causing immune impairment	3200	<ul style="list-style-type: none"> 2 parts (Phase I: sentinel safety cohort and Phase III: main cohort) Phase I (sentinel safety cohort): 56 healthy adults, age 18 to 55 years, randomised in a 5:2 ratio to receive AZD5156 or placebo Phase III (main cohort): randomised 1:1 to receive AZD3152 300mg or comparator (600mg <i>Evusheld</i> or placebo) administered i.m. in the anterolateral thigh on Day 1; participants will receive a second dose of their original randomised trial intervention 6 months after Visit 1 Phase II (sub-study, open-label): participants randomised 2:1 to receive 1200mg i.v. AZD3152 or 300mg i.m. <i>Evusheld</i> Global trial 	<ul style="list-style-type: none"> Primary endpoints (Phase III main cohort): to evaluate the safety of AZD3152 and <i>Evusheld</i> and/or placebo and to compare the efficacy of AZD3152 to <i>Evusheld</i> and/or placebo in the prevention of symptomatic COVID-19 Primary endpoints (Phase II sub-study): to evaluate the safety of AZD3152 and <i>Evusheld</i>; to compare the nAb responses to the SARS-CoV-2 to a current variant of concern following AZD3152 administration vs. SARS-CoV-2 nAb responses to prior variants following <i>Evusheld</i> administration, to characterise the PK of AZD3152 and <i>Evusheld</i> in serum and to evaluate the ADA responses to AZD3152 and AZD7442 in serum 	<ul style="list-style-type: none"> FPCD: Q4 2022 Data anticipated: H2 2023
Phase I LITTLE DIPPER NCT05872958	Healthy adult participants; age 18 to 55 years	96	<ul style="list-style-type: none"> Phase I, double-blind, placebo-controlled, multi-centre, dose exploration study to evaluate the safety and PK of AZD3152 in healthy adult participants across different dose levels and routes of administration Approximately 96 participants randomised in a 10:2 ratio to receive either AZD3152 or placebo administered i.m. or i.v. across 5 fixed-dose cohorts 	<ul style="list-style-type: none"> Primary endpoint: to evaluate the safety of i.m. or i.v. administration of AZD3152 and to characterise the PK of AZD3152 in serum after a single i.m. or i.v. dose Secondary endpoint: to evaluate ADA responses to AZD3152 	<ul style="list-style-type: none"> FPCD: Q2 2023 LPCD: Q3 2023 Data readout: Q4 2023



BioPharmaceuticals: early-stage development



AZD0780 (PCSK9 inhibitor)

Dyslipidaemia

Approved medicines

Late-stage development

Early development

Trial	Population	Patients	Design	Endpoints	Status
Phase I NCT05384262	Healthy adults	132	<ul style="list-style-type: none">Randomised, placebo-controlled SAD/MAD trial	<ul style="list-style-type: none">Primary endpoints: safety and tolerability	<ul style="list-style-type: none">FPCD: Q2 2022Data anticipated: H1 2024
Phase I NCT05787002	Healthy volunteers	16	<ul style="list-style-type: none">Open-label, two-period, two-sequence crossover study to assess the effect of AZD0780 on the PK of <i>Crestor</i>	<ul style="list-style-type: none">Primary endpoints: PK parameters, safety and tolerability	<ul style="list-style-type: none">FPCD: Q1 2023LPCD: Q2 2023Data anticipated: H2 2023
Phase I NCT05817461	Healthy volunteers	8	<ul style="list-style-type: none">Open-label, two-part sequential human ADME study	<ul style="list-style-type: none">Primary endpoints: mass balance recovery, absorption, metabolism, excretion of [14C]AZD0780 and absolute bioavailability of AZD0780Secondary endpoints: safety and tolerability	<ul style="list-style-type: none">FPCD: Q2 2022LPCD: Q2 2022Data anticipated: H2 2023

Oncology

CVRM

R&I

Other

V&I

Rare Disease



AZD2373

Chronic kidney disease

Approved medicines

Late-stage development

Early development

Oncology

CVRM

R&I

Other

V&I

Rare Disease

Trial	Population	Patients	Design	Endpoints	Status
Phase I NCT04269031	Healthy volunteers	30	<ul style="list-style-type: none"> SAD dose escalation in 6 cohorts with 6 volunteers receiving AZD2373 and 2 volunteers receiving placebo in each cohort Arm 1: AZD2373 s.c. Arm 2: placebo s.c. US only 	<ul style="list-style-type: none"> Primary endpoints: safety and tolerability Secondary endpoint: PK parameters 	<ul style="list-style-type: none"> FPCD: Q1 2020 LPCD: Q3 2021 Data readout: Q3 2022
Phase I NCT05351047	Healthy volunteers	24	<ul style="list-style-type: none"> MAD dose escalation in 3 cohorts with 6 volunteers per cohort receiving AZD2373 and 2 volunteers per cohort receiving placebo Arm 1: AZD2373 s.c. Arm 2: placebo s.c. US only 	<ul style="list-style-type: none"> Primary endpoints: safety and tolerability Secondary endpoints: PK parameters, effect of s.c. MAD administrations of AZD2373 on plasma concentrations of APOL1 protein and APOL1 G0, G1, G2 allele genotype status in trial participants 	<ul style="list-style-type: none"> FPCD: Q2 2022 LPCD: Q1 2023 Data anticipated: H2 2023



AZD2693 (antisense oligonucleotide)

NASH

Trial	Population	Patients	Design	Endpoints	Status
Phase IIb FORTUNA NCT05809934	NASH with fibrosis	180	<ul style="list-style-type: none"> Randomised, double-blind, placebo-controlled, multi-centre trial Arm 1: AZD2693 s.c. dose 1 Arm 2: AZD2693 s.c. dose 2 Arm 3: placebo s.c. Global trial 	<ul style="list-style-type: none"> Primary endpoints: efficacy, safety and tolerability of AZD2693 	<ul style="list-style-type: none"> FPCD: Q2 2023 Data anticipated: >2024
Phase I NCT04142424	Healthy volunteers	72	<ul style="list-style-type: none"> SAD with 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 only 	<ul style="list-style-type: none"> Primary endpoints: safety and tolerability Secondary endpoint: PK parameters 	<ul style="list-style-type: none"> FPCD: Q4 2019 LPCD: Q3 2021 Data readout: Q1 2022
Phase I NCT04483947	NASH/NAFLD F0-F3	74	<ul style="list-style-type: none"> MAD with 4 cohorts receiving AZD2693 and placebo in each cohort Arm 1: AZD2693 s.c. Arm 2: placebo s.c. US only 	<ul style="list-style-type: none"> Primary endpoints: safety and tolerability Secondary endpoint: PK parameters 	<ul style="list-style-type: none"> FPCD: Q2 2021 Data anticipated: H1 2024
Phase I NCT05107336	Healthy volunteers	44	<ul style="list-style-type: none"> MAD with 4 cohorts receiving AZD2693 and placebo in each cohort Arm 1: AZD2693 s.c. Arm 2: placebo s.c. JP only 	<ul style="list-style-type: none"> Primary endpoints: safety and tolerability Secondary endpoint: PK parameters 	<ul style="list-style-type: none"> FPCD: Q4 2021 LPCD: Q4 2022 Data anticipated: H2 2023
Phase I NCT05919069	Hepatic impairment	32	<ul style="list-style-type: none"> Single-dose, non-randomised, open-label, parallel group study US only 	<ul style="list-style-type: none"> Primary endpoints: safety, tolerability and PK parameters 	<ul style="list-style-type: none"> FPCD: Q3 2023 Data anticipated: H2 2024



AZD3427 (relaxin)

Heart failure

Approved medicines

Late-stage development

Early development

Oncology

CVRM

R&I

Other

V&I

Rare Disease

Trial	Population	Patients	Design	Endpoints	Status
Phase II Re-PHiRE NCT05737940	Heart failure and pulmonary hypertension due to left heart disease	220	<ul style="list-style-type: none">• Randomised, double-blind, placebo-controlled, multi-centre trial• Arm 1: AZD3427 (high dose)• Arm 2: AZD3427 (medium dose)• Arm 3: AZD3427 (low dose)• Arm 4: placebo• Global trial – US, Canada, China, Japan, Czech Republic, Italy, Spain, Netherlands, Poland, UK, Austria, Germany, Denmark and Sweden	<ul style="list-style-type: none">• Primary endpoint: change in PVR from baseline to Week 25 vs. placebo as measured by right heart catheterisation	<ul style="list-style-type: none">• FPCD: Q2 2023• Data anticipated: >2024



AZD5462 (relaxin)

Heart failure

Approved medicines

Late-stage development

Early development

Oncology

CVRM

R&I

Other

V&I

Rare Disease

Trial	Population	Patients	Design	Endpoints	Status
Phase I NCT04994106	Healthy volunteers (SAD/MAD)	98	<ul style="list-style-type: none">• Single-centre SAD and MAD• Part A: SAD (8 cohorts)• Arm 1: AZD5462• Arm 2: placebo• Part B: MAD (5 cohorts)• Arm 1: AZD5462• Arm 2: placebo• US only	<ul style="list-style-type: none">• Primary endpoints: safety and tolerability	<ul style="list-style-type: none">• FPCD: Q4 2021• LPCD: Q3 2022• Data readout: Q2 2023



AZD6234 (long-acting amylin)

Obesity with related comorbidities

Trial	Population	Patients	Design	Endpoints	Status
Phase I NCT05511025	Healthy participants who are overweight or obese	64	<ul style="list-style-type: none">SAD trial	<ul style="list-style-type: none">Primary endpoint: safety	<ul style="list-style-type: none">FPCD: Q4 2022Data anticipated: H1 2024



AZD7503 (antisense oligonucleotide)

NASH

Trial	Population	Patients	Design	Endpoints	Status
Phase I NCT05143905	Healthy volunteers	56	<ul style="list-style-type: none"> SAD, 7 cohorts Arm 1: AZD7503 s.c. Arm 2: placebo s.c. US only 	<ul style="list-style-type: none"> Primary endpoints: safety and tolerability Secondary endpoint: PK 	<ul style="list-style-type: none"> FPCD: Q4 2021 Data anticipated: H2 2023
Phase I NCT05560607	NAFLD or NASH	14	<ul style="list-style-type: none"> Single-centre, open-label Phase I study to assess knockdown of hepatic HSD17B13 mRNA PK, safety and tolerability following multiple doses of AZD7503 	<ul style="list-style-type: none"> Primary endpoint: safety and tolerability Secondary endpoint: change in HSD17B13 mRNA expression 	<ul style="list-style-type: none"> FPCD: Q3 2022 Data anticipated: H2 2024
Phase I NCT05864391	NASH F1-F3	60	<ul style="list-style-type: none"> Randomised, single-blind, MAD trial 	<ul style="list-style-type: none"> Primary endpoint: safety and tolerability Secondary endpoint: PK parameters 	<ul style="list-style-type: none"> FPCD: Q3 2023 Data anticipated: H2 2024



AZD9550 (GLP-1-glucagon agonist)

NASH

Approved medicines

Late-stage development

Early development

Oncology

CVRM

R&I

Other

V&I

Rare Disease

Trial	Population	Patients	Design	Endpoints	Status
Phase I NCT05848440	Healthy volunteers	64	<ul style="list-style-type: none">SAD trial	<ul style="list-style-type: none">Primary endpoint: safety and tolerability	<ul style="list-style-type: none">FPCD: Q2 2023Data anticipated: H1 2024



balcinrenone/dapagliflozin (MR modulator + SGLT2 inhibitor)

Heart failure

Trial	Population	Patients	Design	Endpoints	Status
Phase IIb MIRACLE NCT04595370	Heart failure with chronic kidney disease	500	<ul style="list-style-type: none"> Randomised, stratified according to T2DM and eGFR (≥ 20 to < 30 mL/min / ≥ 30 to < 45 mL/min / ≥ 45 mL/min) for 12 weeks Arm 1: AZD9977 A + <i>Farxiga</i> 10mg Arm 2: AZD9977 B + <i>Farxiga</i> 10mg Arm 3: AZD9977 C + <i>Farxiga</i> 10mg Arm 4: <i>Farxiga</i> 10mg 12 weeks Global trial – 19 countries 	<ul style="list-style-type: none"> 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 <i>Farxiga</i> and 3 doses of AZD9977 combined with <i>Farxiga</i> on UACR; safety, tolerability and serum potassium values; eGFR 	<ul style="list-style-type: none"> FPCD: Q2 2021 Data anticipated: H2 2023



MEDI6570

Cardiovascular

Approved medicines

Late-stage development

Early development

Oncology

CVRM

R&I

Other

V&I

Rare Disease

Trial	Population	Patients	Design	Endpoints	Status
Phase IIb NCT04610892	Post-myocardial infarction	400	<ul style="list-style-type: none"> Evaluation of anti-inflammatory potential and effect on surrogates for atherosclerotic and heart failure events Arm 1: MEDI6570 (high dose) Arm 2: MEDI6570 (medium dose) Arm 3: MEDI6570 (low dose) Arm 4: placebo Global trial – US, Canada, Hungary, Japan, Czech Republic, Italy, Spain, Netherlands, Poland, UK, Australia and Russia 	<ul style="list-style-type: none"> Primary endpoints: safety and efficacy 	<ul style="list-style-type: none"> FPCD: Q4 2020 LPCD: Q4 2022 Data anticipated: H1 2024



MEDI8367

Chronic kidney disease

Approved medicines

Late-stage development

Early development

Oncology

CVRM

R&I

Other

V&I

Rare Disease

Trial	Population	Patients	Design	Endpoints	Status
Phase I NCT04365218	Healthy volunteers	12	<ul style="list-style-type: none">• SAD trial• 6 cohorts• Arm 1: MEDI8367 s.c.• Arm 2: placebo s.c.• US only	<ul style="list-style-type: none">• Primary endpoints: safety and tolerability• Secondary endpoints: PK parameters and ADA	<ul style="list-style-type: none">• FPCD: Q3 2020• LPCD: Q4 2020• Data readout: Q2 2022



mitiperstat (MPO inhibitor)

Cardiovascular disease

Approved medicines

Late-stage development

Early development

Oncology

CVRM

R&I

Other

V&I

Rare Disease

Trial	Population	Patients	Design	Endpoints	Status
Phase I NCT05236543	Healthy volunteers	14	<ul style="list-style-type: none">Open-labelmitiperstat vs. mitiperstat and itraconazoleUK only	<ul style="list-style-type: none">Primary endpoints: PK parametersSecondary endpoints: safety and tolerability	<ul style="list-style-type: none">FPCD: Q1 2022LPCD: Q3 2022Data readout: Q1 2023
Phase I NCT05457270	Healthy volunteers	30	<ul style="list-style-type: none">Open-label2-period, 2-treatment, single-dose, crossover trialPeriod 1: single oral dose mitiperstat Formulation A or B on Day 1Period 2: single oral dose mitiperstat Formulation A or B on Day 1US only	<ul style="list-style-type: none">Primary endpoints: relative bioavailability and PK parametersSecondary endpoints: safety and tolerability	<ul style="list-style-type: none">FPCD: Q3 2022LPCD: Q3 2022Data readout: Q1 2023



mitiperstat (MPO inhibitor)

NASH

Approved medicines

Late-stage development

Early development

Oncology

CVRM

R&I

Other

V&I

Rare Disease

Trial	Population	Patients	Design	Endpoints	Status
Phase II COSMOS NCT05638737	NASH	90	<ul style="list-style-type: none">• Randomised, placebo-controlled, double-blind• Arm 1: 5mg mitiperstat• Arm 2: placebo• Global trial	<ul style="list-style-type: none">• Primary endpoints: safety, tolerability and PD parameters	<ul style="list-style-type: none">• FPCD: Q1 2023• Data anticipated: H2 2024
Phase I NCT05751759	Participants with hepatic impairment and participants with normal hepatic function	32	<ul style="list-style-type: none">• Phase I, single dose, non-randomised, open-label, parallel-group trial	<ul style="list-style-type: none">• Primary endpoints: safety, tolerability and PK parameters	<ul style="list-style-type: none">• FPCD: Q1 2023• Data anticipated: H2 2024



atuliflapon (FLAP inhibitor)

Asthma

Approved medicines

Late-stage development

Early development

Oncology

CVRM

R&I

Other

V&I

Rare Disease

Trial	Population	Patients	Design	Endpoints	Status
Phase II FLASH NCT05251259	Patients with moderate-to-severe uncontrolled asthma	1102	<ul style="list-style-type: none">• Randomised, placebo-controlled, double-blind, multi-centre, 2-part trial with a lead-in PK cohort• PK cohort• Arm 1: atuliflapon• Arm 2: placebo• Part 1• Arm 1: atuliflapon• Arm 2: placebo• Part 2• Arm 1: atuliflapon dose A• Arm 2: atuliflapon dose B• Arm 3: atuliflapon dose C• Arm 4: placebo• Global trial	<ul style="list-style-type: none">• Primary endpoint: time to first CompEx asthma event	<ul style="list-style-type: none">• FPCD: Q2 2022• Data anticipated: >2024



AZD4604 (inhaled JAK-1 inhibitor)

Asthma

Trial	Population	Patients	Design	Endpoints	Status
Phase I NCT04769869	Healthy volunteers and patients with mild asthma	137	<ul style="list-style-type: none"> SAD/MAD/POM trial Part 1 SAD Arm 1: AZD4604 (DPI) Arm 2: placebo (DPI) Part 2 MAD Arm 1: AZD4604 (DPI) Arm 2: placebo (DPI) Part 3 POM Arm 1: AZD4604 (DPI) Arm 2: placebo (DPI) UK only 	<ul style="list-style-type: none"> Primary endpoints: safety and tolerability Secondary endpoints: PK parameters and FENO 	<ul style="list-style-type: none"> FPCD: Q4 2021 Data readout: Q3 2023



AZD5055 (oral porcupine inhibitor)

IPF and other ILDs with progressive fibrosis

Trial	Population	Patients	Design	Endpoints	Status
Phase I NCT05134727	Healthy volunteers	90	<ul style="list-style-type: none"> SAD/MAD trial Part 1: SAD Arm 1: AZD5055 (oral suspension) Arm 2: placebo (oral suspension) Part 2: MAD Arm 1: AZD5055 (oral suspension) Arm 2: placebo (oral suspension) 	<ul style="list-style-type: none"> Primary endpoints: safety and tolerability Secondary endpoints: PK parameters 	<ul style="list-style-type: none"> FPCD: Q4 2021 LPCD: Q2 2023 Data readout: Q2 2023
Phase I NCT05630677	Healthy volunteers	18	<ul style="list-style-type: none"> BA trial to compare film-coated tablet with oral suspension and to assess the effect of food and an acid reducing agent on PK of AZD5055 in healthy volunteers 	<ul style="list-style-type: none"> Primary endpoints: bioavailability and PK parameters 	<ul style="list-style-type: none"> FPCD: Q4 2022 LPCD: Q1 2023 Data readout: Q2 2023



AZD6793 (IRAK4)

Inflammatory diseases

Approved medicines

Late-stage development

Early development

Oncology

CVRM

R&I

Other

V&I

Rare Disease

Trial	Population	Patients	Design	Endpoints	Status
Phase I NCT05662033	Healthy volunteers	133	<ul style="list-style-type: none">Single-blind, randomised, placebo-controlled study to investigate the safety, tolerability and PK of oral AZD6793 following single and multiple ascending doses in healthy subjects	<ul style="list-style-type: none">Primary endpoints: safety and tolerabilitySecondary endpoint: PK parameters	<ul style="list-style-type: none">FPCD: Q4 2022Data anticipated: H1 2024



AZD7798 (humanised mAb)

Crohn's disease

Approved medicines

Late-stage development

Early development

Oncology

CVRM

R&I

Other

V&I

Rare Disease

Trial	Population	Patients	Design	Endpoints	Status
Phase I NCT05452304	Healthy volunteers	96	<ul style="list-style-type: none">SADArm1: AZD7798Arm2: placebo	<ul style="list-style-type: none">Primary endpoints: safety and tolerabilitySecondary endpoints: PK parameters and immunogenicity	<ul style="list-style-type: none">FPCD: Q3 2022Data anticipated: H2 2023



AZD8630 (inhaled TSLP)

Asthma

Approved medicines

Late-stage development

Early development

Oncology

CVRM

R&I

Other

V&I

Rare Disease

Trial	Population	Patients	Design	Endpoints	Status
Phase I NCT05110976 Partnered (AMGEN)	Healthy volunteers and patients with asthma	232	<ul style="list-style-type: none">SAD and MAD trial	<ul style="list-style-type: none">Primary endpoints: safety and tolerabilitySecondary endpoints: PK parameters and FENO	<ul style="list-style-type: none">FPCD: Q1 2022LPCD: Q3 2023Data readout: Q4 2023



mitiperstat (MPO inhibitor)

COPD

Approved medicines

Late-stage development

Early development

Oncology

CVRM

R&I

Other

V&I

Rare Disease

Trial	Population	Patients	Design	Endpoints	Status
Phase II CRESCENDO NCT05492877	Moderate to severe COPD; age 40 to 80	288	<ul style="list-style-type: none">• Randomised, double-blind trial• Arm 1: 5mg mitiperstat• Arm 2: placebo• Global trial – 14 countries	<ul style="list-style-type: none">• Primary endpoint: time to first COPD CompEx event• Secondary endpoints: plasma concentration-time profiles, PK parameters, time to first COPD exacerbation event, post-BD FEV1, respiratory symptoms, disease impact, safety and tolerability	<ul style="list-style-type: none">• FPCD: Q1 2023• Data anticipated: H1 2024



AZD4041 (orexin 1 receptor antagonist)

Opioid use disorder

Trial	Population	Patients	Design	Endpoints	Status
Phase I NCT05587998 Partnered (National Institute on Drug Abuse)	Healthy recreational opioid users	36	<ul style="list-style-type: none"> Randomised, double-blind, placebo-controlled, fixed sequence trial 	<ul style="list-style-type: none"> Primary endpoint: change in respiratory parameters 	<ul style="list-style-type: none"> FPCD: Q3 2022 LPCD: Q2 2023 Data readout: Q3 2023 Primary endpoint met



MEDI0618 (PAR2 antagonist mAb)

Osteoarthritis pain, migraine prevention

Trial	Population	Patients	Design	Endpoints	Status
Phase I NCT04198558	Healthy volunteers	64	<ul style="list-style-type: none"> SAD trial Arm 1: MEDI0618 i.v. Arm 2: placebo i.v. Arm 3: MEDI0618 s.c. Arm 4: placebo s.c. Europe only 	<ul style="list-style-type: none"> Primary endpoints: safety and tolerability Secondary endpoint: PK parameters 	<ul style="list-style-type: none"> FPCD: Q4 2019 LPCD: Q1 2022 Data readout: Q2 2022
Phase I NCT05714254 (.)	Healthy volunteers	48	<ul style="list-style-type: none"> Randomised, double-blind, placebo-controlled MAD trial Arm 1: MEDI0618 i.v. or placebo Arm 2: MEDI0618 s.c. or placebo 	<ul style="list-style-type: none"> Primary endpoints: safety, tolerability and PK parameters 	<ul style="list-style-type: none"> FPCD: Q4 2022 LPCD: Q3 2023 Data anticipated: H1 2024



MEDI1341 (alpha-synuclein mAb)

Multiple system atrophy

Trial	Population	Patients	Design	Endpoints	Status
Phase II NCT05526391 Partnered (Takeda)	Patients with diagnosis of possible or probably MSA (using modified Gilman et al. 2008 diagnostic criteria)	138	<ul style="list-style-type: none"> Randomised, double-blind, placebo-controlled trial Early PK cohort Arm 1: TAK-341/MEDI1341 i.v. Arm 2: placebo i.v. Main cohort Arm 3: TAK-341/MEDI1341 i.v. Arm 4: placebo i.v. 	<ul style="list-style-type: none"> Primary endpoints: efficacy, change from baseline on modified Unified Multiple System Atrophy Rating Scale at 52 weeks Secondary endpoints: PK parameters, safety and efficacy 	<ul style="list-style-type: none"> FPCD: Q4 2022 Data anticipated: >2024



MEDI1341 (alpha-synuclein mAb)

Parkinson's disease

Approved medicines

Late-stage development

Early development

Oncology

CVRM

R&I

Other

V&I

Rare Disease

Trial	Population	Patients	Design	Endpoints	Status
Phase I NCT04449484 Partnered (Takeda)	Parkinson's disease	25	<ul style="list-style-type: none">• MAD trial• Arm 1: MEDI1341 i.v.• Arm 2: placebo i.v.• US only	<ul style="list-style-type: none">• Primary endpoints: safety and tolerability• Secondary endpoints: PK and PD parameters	<ul style="list-style-type: none">• FPCD: Q3 2020• LPCD: Q3 2021• Data readout: Q4 2022



MEDI7352 (NGF TNF bispecific mAb)

Osteoarthritis pain

Trial	Population	Patients	Design	Endpoints	Status
Phase IIb NCT04675034	Painful osteoarthritis of the knee	350	<ul style="list-style-type: none"> MAD trial Arm 1: MEDI7352 s.c. Arm 2: placebo s.c. Global – 7 countries 	<ul style="list-style-type: none"> Primary endpoint: dose response Secondary endpoints: safety, tolerability, PK and PD parameters, ADA 	<ul style="list-style-type: none"> FPCD: Q1 2021 LPCD: Q3 2022 Data anticipated: H2 2023
Phase IIa NCT03755934	Painful diabetic neuropathy	107	<ul style="list-style-type: none"> MAD trial Arm 1: MEDI7352 i.v. Arm 2: placebo i.v. Europe only 	<ul style="list-style-type: none"> Primary endpoint: dose response Secondary endpoints: safety, tolerability, PK and PD parameters 	<ul style="list-style-type: none"> FPCD: Q4 2018 LPCD: Q1 2023 Data anticipated: H2 2023



**Rare Disease: approved
medicines and late-stage
pipeline**



Koselugo (selumetinib, MEK inhibitor)

Neurofibromatosis type 1, solid tumours

Trial	Population	Patients	Design	Endpoints	Status
Phase III KOMET NCT04924608	Adult age ≥18 years with NF1 who have symptomatic, inoperable PN Available baseline chronic target PN pain score	146	<ul style="list-style-type: none"> Multi-centre, international trial with a parallel, randomised, double-blind, placebo-controlled, 2 arm design Arm 1: <i>Koselugo</i> 25mg/m² BID Arm 2: placebo BID until end of Cycle 12, then crossover to <i>Koselugo</i> 25mg/m² BID 	<ul style="list-style-type: none"> Primary endpoint: ORR by end of Cycle 16 on <i>Koselugo</i> vs. placebo as determined by ICR per REiNS criteria Secondary endpoint: change in baseline of chronic PN-pain intensity on <i>Koselugo</i> vs. placebo 	<ul style="list-style-type: none"> FPCD: Q4 2021 Data anticipated: H2 2024
Phase I/II SPRINKLE NCT05309668	Paediatric (age 1 to 6 years) diagnosed with NF1 with symptomatic, inoperable PN with at least one measurable PN, defined as a PN of at least 3cm, measured in one dimension	38	<ul style="list-style-type: none"> Single-arm, open-label with <i>Koselugo</i> 	<ul style="list-style-type: none"> Primary endpoints: <i>Koselugo</i> AUC₀₋₁₂ derived after single dose administration [time frame: pre-dose and 1, 2, 3, 4, 6, 8 and 10-12 hours after selumetinib single dose on the first day of treatment (Cycle 1 Day 1)]; AEs graded by CTCAE Ver 5.0 [time frame: from screening until 30 days after last dose] 	<ul style="list-style-type: none"> FPCD: Q1 2022 Data anticipated: H1 2024
Phase I China PK/Safety/Efficacy NCT04590235	Pediatric (age 2 to 17 years old), adult NF1	32	<ul style="list-style-type: none"> Single-arm trial with 3 phases: dose confirmation phase (n=6 for 3 cycles), expansion phase (24 months post-LSD) and long-term follow-up (60 months post-LSD) 	<ul style="list-style-type: none"> Primary endpoints: safety, tolerability and PK parameters Secondary endpoint: efficacy (ORR, DoR; TTR; PFS) 	<ul style="list-style-type: none"> FPCD: Q1 2021 Data anticipated: H2 2023
Phase I Food Effect/GI Tolerability NCT05101148	Adolescents aged ≥12 to <18 years at trial entry with a clinical diagnosis of NF1-related PN <i>Koselugo</i> with a low-fat meal compared to fasted state	24	<ul style="list-style-type: none"> Single-arm, multiple dose, sequential, two or three period trial <i>Koselugo</i> 25mg/m² BID given with a low-fat meal vs. the same dose given in a fasted state 	<ul style="list-style-type: none"> Primary endpoints: PK parameters (steady state systemic exposure), safety (GI toxicity) 	<ul style="list-style-type: none"> FPCD: Q3 2021 Data anticipated: H2 2023



Ultomiris (anti-C5 mAb)

Haematology, nephrology

Trial	Population	Patients	Design	Endpoints	Status
Phase III ALXN1210-TM-313 NCT04543591	Thrombotic microangiopathy-associated haematopoietic stem cell transplant	106	<ul style="list-style-type: none"> Arm 1: <i>Ultomiris</i> Q8W Arm 2: placebo 	<ul style="list-style-type: none"> Primary endpoint: TMA response Secondary endpoints: time to TMA response, TMA relapse 	<ul style="list-style-type: none"> FPCD: Q4 2020 Data anticipated: >2024
Phase III ALXN1210-TM-314 NCT04557735	Paediatric thrombotic microangiopathy-associated haematopoietic stem cell transplant	40	<ul style="list-style-type: none"> Arm 1: <i>Ultomiris</i> administered once every 4 to 8 weeks 	<ul style="list-style-type: none"> Primary endpoint: proportion of participants with TMA response Secondary endpoints: time to TMA response, proportion of participants with TMA relapse 	<ul style="list-style-type: none"> FPCD: Q4 2020 Data anticipated: >2024
Phase III ARTEMIS (ALXN1210-CSA-AKI-318) NCT05746559	CSA-AKI	736	<ul style="list-style-type: none"> Randomised, double-blind, placebo-controlled, multicentre trial <i>Ultomiris</i> i.v. to protect patients with CKD from CSA-AKI and subsequent MAKE 	<ul style="list-style-type: none"> Primary endpoint: to assess the efficacy of a single dose of <i>Ultomiris</i> i.v. vs. placebo in reducing the risk of the clinical consequences of AKI (MAKE) at 90 days in adult participants with CKD who undergo non-emergent cardiac surgery with CPB 	<ul style="list-style-type: none"> FPCD: Q1 2023 Data anticipated: >2024
Phase II SANCTUARY (ALXN1210-NEPH-202) NCT04564339	Proliferative lupus nephritis or immunoglobulin A nephropathy	120	<ul style="list-style-type: none"> Arm 1: LN cohort, <i>Ultomiris</i> Arm 2: LN cohort, placebo Arm 3: IgAN cohort, <i>Ultomiris</i> Arm 4: IgAN cohort, placebo 	<ul style="list-style-type: none"> Primary endpoint: percentage change in proteinuria from baseline to Week 26 Secondary endpoints: percentage change in proteinuria from baseline to Week 50 	<ul style="list-style-type: none"> FPCD: Q1 2021 Data anticipated: >2024 Primary endpoint met (IgAN cohort)



Ultomiris (anti-C5 mAb)

Neurology

Trial	Population	Patients	Design	Endpoints	Status
Phase III ALXN1210-NMO-307 NCT04201262	Neuromyelitis optica spectrum disorder	58	• Arm 1: <i>Ultomiris</i> Q8W	• Primary endpoint: time to first adjudicated on-trial relapse	• FPCD: Q4 2019 • LPCD: Q1 2021 • Data readout: Q2 2022 • Primary endpoint met
Phase II/III ALXN1210-DM-310 NCT04999020	Dermatomyositis	150	• Arm 1: <i>Ultomiris</i> • Arm 2: placebo	• Primary endpoint: improvement response on IMACS-TIS	• FPCD: Q4 2021 • Data anticipated: H1 2024
Phase II/III ALXN1210-NMO-317 NCT05346354	Neuromyelitis optica spectrum disorder	12	• Arm 1: <i>Ultomiris</i> Q8W	• Primary endpoint: change from baseline in annualised relapse rate at Week 50	• FPCD: Q3 2022 • Data anticipated: >2024



acoramidis (ALXN2060)

ATTR-CM

Approved medicines

Late-stage development

Early development

Trial	Population	Patients	Design	Endpoints	Status
Phase III ALXN2060-TAC-302 NCT04622046	ATTR-CM	22	<ul style="list-style-type: none">• Arm 1: 800mg acoramidis administered twice daily• Japan only	<ul style="list-style-type: none">• Primary endpoint: change from baseline to Month 12 of treatment in distance walked during the six-minute walk test, cause mortality and cardiovascular related hospitalisation over a 30-month period	<ul style="list-style-type: none">• FPCD: Q4 2020• Data anticipated: H1 2024

Oncology

CVRM

R&I

Other

V&I

Rare Disease



anselamimab (CAEL-101, fibril-reactive mAb)

AL amyloidosis

Trial	Population	Patients	Design	Endpoints	Status
Phase III CAEL101-302 NCT04512235	AL amyloidosis (Mayo Stage IIIa)	267	<ul style="list-style-type: none"> Arm 1: anselamimab combined with SoC for PCD Arm 2: placebo combined with SoC for PCD 	<ul style="list-style-type: none"> Primary endpoint: time from first dose of trial drug until death or end of trial Secondary endpoint: change in distance walked during a six-minute walk test and quality of life measures 	<ul style="list-style-type: none"> FPCD: Q4 2020 Data anticipated: H2 2024
Phase III CAEL101-301 NCT04504825	AL amyloidosis (Mayo Stage IIIb)	124	<ul style="list-style-type: none"> Arm 1: anselamimab combined with SoC for PCD Arm 2: placebo combined with SoC for PCD 	<ul style="list-style-type: none"> Primary endpoint: time from first dose of trial drug until death or end of trial Secondary endpoint: change in distance walked during a six-minute walk test and quality of life measures 	<ul style="list-style-type: none"> FPCD: Q1 2021 Data anticipated: >2024
Phase II CAEL101-203 NCT04304144	AL amyloidosis (Mayo Stage I, Stage II and Stage IIIa)	25	<ul style="list-style-type: none"> Arm 1: anselamimab combined with SoC CyBorD Arm 2: placebo combined with SoC CyBorD and daratumumab 	<ul style="list-style-type: none"> Primary endpoint: occurrence of DLT during the first 4 weeks of therapy Secondary endpoint: AUC (plasma curve concentration) 	<ul style="list-style-type: none"> FPCD: Q1 2020 Data anticipated: H1 2024



danicopan (ALXN2040, factor D inhibitor)

Haematology, ophthalmology

Trial	Population	Patients	Design	Endpoints	Status
Phase III ALXN2040-PNH-301 NCT04469465	PNH with clinically significant EVH	86	<ul style="list-style-type: none"> Arm 1: danicopan + C5 Inhibitor Arm 2: placebo + C5 Inhibitor 	<ul style="list-style-type: none"> Primary endpoint: change from baseline in haemoglobin at Week 12 Secondary endpoint: percentage of participants with transfusion avoidance 	<ul style="list-style-type: none"> FPCD: Q1 2021 Data readout: Q3 2022 Primary endpoint met
Phase III ALXN2040-PNH-303 NCT05389449	PNH	100	<ul style="list-style-type: none"> Arm 1: danicopan together with background C5 inhibitor therapy 	<ul style="list-style-type: none"> Primary endpoint: participants experiencing TEAEs and serious TEAEs 	<ul style="list-style-type: none"> FPCD: Q4 2022 Data anticipated: >2024
Phase II ALXN2040-GA-201 NCT05019521	Geographic atrophy	365	<ul style="list-style-type: none"> Arms 1-3: danicopan dosed at 100mg-400mg QD Arm 4: placebo 	<ul style="list-style-type: none"> 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 	<ul style="list-style-type: none"> FPCD: Q3 2021 Data anticipated: >2024



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

Neurology, nephrology

Approved medicines

Late-stage development

Early development

Trial	Population	Patients	Design	Endpoints	Status
Phase III ALXN1720-MG-301 NCT05556096	Generalised myasthenia gravis	254	<ul style="list-style-type: none"> Arm 1: weight-based maintenance treatment with gefurulimab on Day 1, followed by weight-based maintenance treatment of gefurulimab on Week 1 (Day 8) and Q1W thereafter for a total of 26 weeks Arm 2: placebo 	<ul style="list-style-type: none"> Primary endpoint: change from baseline in MG-ADL total score at Week 26 	<ul style="list-style-type: none"> FPCD: Q4 2022 Data anticipated: >2024
Phase I ALXN1720-NEPH-102 NCT05314231	Proteinuria	13	<ul style="list-style-type: none"> Arm 1: gefurulimab, s.c. infusion at a dose of 1500mg 	<ul style="list-style-type: none"> Primary endpoint: serum concentration of [time frame: Day 1 (0.5 hours pre-dose and post-dose) and dose on Days 2, 3, 8, 15, 29, 43 and 57] 	<ul style="list-style-type: none"> FPCD: Q2 2022 Data readout: Q3 2023

Oncology

CVRM

R&I

Other

V&I

Rare Disease



**Rare Disease:
early-stage development**



ALXN1850 (next-generation asfotase alfa)

Hypophosphatasia

Trial	Population	Patients	Design	Endpoints	Status
Phase I ALXN1850-HPP-101 NCT04980248	Hypophosphatasia	15	<ul style="list-style-type: none"> Arm 1: ALXN1850, 3 cohorts at low, medium and high dosages 	<ul style="list-style-type: none"> Primary endpoint: incidence of TEAEs and TESAEs 	<ul style="list-style-type: none"> FPCD: Q3 2021 Data readout: Q4 2022 Primary endpoint met



ALXN1910 (next-generation TNSALP ERT)

Bone metabolism

Trial	Population	Patients	Design	Endpoints	Status
Phase I ALXN1910-HV-101 NCT05307978	Healthy adults	48	<ul style="list-style-type: none">• Randomised, placebo-controlled SAD	<ul style="list-style-type: none">• Primary endpoint: safety	<ul style="list-style-type: none">• FPCD: Q2 2022• Data readout: Q2 2023



ALXN1920 (kidney-targeted factor H fusion protein)

Nephrology

Approved medicines
Late-stage development
Early development

Trial	Population	Patients	Design	Endpoints	Status
Phase I ALXN1920-HV-101 NCT05751642	Healthy adults	48	<ul style="list-style-type: none">Randomised, double-blind, placebo-controlled, SAD	<ul style="list-style-type: none">Primary endpoint: safety and tolerabilitySecondary endpoints: PK/PD parameters	<ul style="list-style-type: none">FPCD: Q2 2023Data anticipated: H2 2024

Oncology

CVRM

R&I

Other

V&I

Rare Disease



ALXN2030 (siRNA targeting complement C3)

Nephrology

Approved medicines

Late-stage development

Early development

Oncology

CVRM

R&I

Other

V&I

Rare Disease

Trial	Population	Patients	Design	Endpoints	Status
Phase I ALXN2030-HV-101 NCT05501717	Healthy volunteers	48	<ul style="list-style-type: none">Randomised, placebo-controlled SAD	<ul style="list-style-type: none">Primary endpoint: safety	<ul style="list-style-type: none">FPCD: Q4 2022Data anticipated: >2024



ALXN2080 (oral factor D inhibitor)

Complement-mediated disease

Trial	Population	Patients	Design	Endpoints	Status
Phase I ALXN2080-HV-101 NCT05428696	Healthy volunteers	90	<ul style="list-style-type: none"> SAD/MAD trial 	<ul style="list-style-type: none"> Primary endpoints: safety and tolerability, PK and PD parameters 	<ul style="list-style-type: none"> FPCD: Q3 2022 Data readout: Q3 2023



tarperprumig (ALXN1820, anti-properdin)

Haematology

Trial	Population	Patients	Design	Endpoints	Status
Phase IIa PHOENIX (ALXN1820-SCD-201) NCT05565092	SCD	30	<ul style="list-style-type: none"> Randomised, open-label Arm 1: 300mg tarperprumig QW Arm 2: 600mg tarperprumig Q4W Arm 3: 300mg tarperprumig Q8W (optional cohort) 	<ul style="list-style-type: none"> Primary endpoints: TEAEs and SAEs Secondary endpoints: PK parameters 	<ul style="list-style-type: none"> FPCD: Q2 2023 Trial discontinued due to strategic portfolio prioritisation
Phase I ALXN1820-HV-101 NCT04631562	Healthy volunteers	60	<ul style="list-style-type: none"> Arm 1: tarperprumig administered s.c. or i.v., multiple ascending doses Arm 2: placebo 	<ul style="list-style-type: none"> Primary endpoint: participants with TEAEs 	<ul style="list-style-type: none"> FPCD: Q1 2021 Data readout: Q1 2023



vemircopan (ALXN2050, factor D inhibitor)

Haematology, nephrology, neurology

Trial	Population	Patients	Design	Endpoints	Status
Phase II ACH228-110 NCT04170023	PNH	29	<ul style="list-style-type: none"> Arm 1: vemircopan monotherapy with groups including treatment-naïve, C5 inhibitor treatment experienced and patients previously receiving danicopan 	<ul style="list-style-type: none"> Primary endpoint: change in haemoglobin relative to baseline Secondary endpoints: number of participants who have transfusion avoidance and change in LDH relative to baseline 	<ul style="list-style-type: none"> FPCD: Q4 2019 Trial discontinued due to efficacy
Phase II ALXN2050-gMG-201 NCT05218096	Generalised myasthenia gravis	70	<ul style="list-style-type: none"> Arm 1: vemircopan 180mg Arm 2: vemircopan 120mg Arm 3: placebo followed by vemircopan 	<ul style="list-style-type: none"> Primary endpoint: MG-ADL total score reduction of ≥ 2 points in any 4 consecutive weeks during the first 8 weeks and who did not receive rescue therapy 	<ul style="list-style-type: none"> FPCD: Q2 2022 Data anticipated: >2024
Phase II ALXN2050-NEPH-201 NCT05097989	Lupus nephritis or immunoglobulin A nephropathy	126	<ul style="list-style-type: none"> Arm 1 – LN cohort: vemircopan 180mg Arm 2 – LN cohort: vemircopan 120mg Arm 3 – LN cohort: placebo Arm 4 – IgAN cohort: vemircopan 180mg Arm 5 – IgAN cohort: vemircopan 120mg Arm 6 – IgAN cohort: placebo 	<ul style="list-style-type: none"> Primary endpoint (both cohorts): percentage change in proteinuria from baseline to Week 26 	<ul style="list-style-type: none"> FPCD: Q3 2022 Data anticipated: >2024
Phase I ALXN2050-HV-109 NCT05259085	Impaired hepatic function	36	<ul style="list-style-type: none"> Arm 1: mild IHF, 120mg vemircopan BID orally on Days 1 through 3, 120mg orally on the morning of Day 4 Arm 2: moderate IHF, 120mg vemircopan BID orally on Days 1 through 3, 120mg orally on the morning of Day 4 Arm 3: severe IHF, 120mg vemircopan BID orally on Days 1 through 3, 120mg orally on the morning of Day 4 Arm 4: healthy control, 120mg vemircopan BID orally on Days 1 through 3, 120mg orally on the morning of Day 4 	<ul style="list-style-type: none"> Primary endpoint (Arm 1): AUC₀₋₁₂ of plasma vemircopan after steady-state Primary endpoint (Arm 2): AUC_t of plasma vemircopan after steady-state Primary endpoint (Arm 3): C_{max,ss} of vemircopan Primary endpoint (Arm 4): T_{max,ss} following vemircopan 	<ul style="list-style-type: none"> FPCD: Q2 2022 Data anticipated: H1 2024



List of abbreviations

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



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	Pyrrrolbenzodiazepine
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
R&I	Respiratory and Immunology
SABA	Short-acting beta2-agonist
SAD	Single ascending dose
SAE	Serious adverse event



List of abbreviations

SBRT	Stereotactic body radiation therapy
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
V&I	Vaccine & Immune therapies
YTE	Triple-amino-acid (M252Y/S254T/T256E [YTE]) substitution

