



Clinical trials appendix

H1 2022 results update



Movement since Q1 2022 update

New to Phase I	New to Phase II	New to Pivotal trial	New to registration
<p><u>NME</u> ALXN1910 Next Gen TNSALP ERT Bone Metabolism</p> <p>AZD0780 PCSK9 dyslipidemia</p> <p>AZD8853 GDF-15 solid tumours</p>	<p><u>Additional indication</u> ALXN2050 Oral Factor D Inhibitor generalized myasthenia gravis</p>	<p><u>Additional indication</u> capivasertib + docetaxel CAPitello-280[#] AKT inhibitor + Docetaxel mCRPC prostate cancer</p> <p>datopotamab deruxtecan TROPION-Breast02[#] TROP-2 targeting antibody drug conjugate 1L TNBC</p> <p><u>Life-cycle management</u> Saphnelo[#] Type I IFN receptor mAb lupus nephritis</p> <p>Tagrisso ADAURA2 adjuvant EGFRm non-small cell lung cancer stage Ia2-Ia3 following complete tumour resection</p>	<p><u>NME</u> PT027^{#1} ICS/SABA asthma</p> <p><u>Life-cycle management</u> Enhertu DESTINY-Breast04^{#1} HER2 targeting antibody drug conjugate HER2-low, unresectable and/or metastatic breast cancer subjects</p>
Removed from Phase I	Removed from Phase II	Removed from Phase III	Removed from registration
<p><u>NME</u> AZD7648[#] DNAPK solid tumours</p> <p>Imfinzi + adavosertib[#] PD-L1 mAb + Wee1 inhibitor solid tumours</p>	<p><u>NME</u> adavosertib[#] Wee1 inhibitor ovarian cancer, solid tumours, uterine serous cancer</p> <p>AZD8601[#] VEGF-A cardiovascular disease</p> <p><u>Life-cycle management</u> Fasenra HILLIER IL-5R mAb atopic dermatitis</p>	<p><u>Life-cycle management</u> Lynparza LYNK-003[#] PARP inhibitor platinum sensitive 1st-line colorectal cancer</p>	<p><u>Life-cycle management</u> Enhertu DESTINY-Breast03^{#2} HER2 targeting antibody drug conjugate HER2-positive, unresectable and/or metastatic breast cancer subjects previously treated with trastuzumab and taxane</p> <p>Ultomiris² anti-complement C5 mAb generalised myasthenia gravis</p>



Q2 2022 Oncology new molecular entity¹ pipeline

Phase 1 13 New Molecular Entities		Phase 2 15 New Molecular Entities		Phase 3 19 New Molecular Entities	
AZD0466# BCL2/xL haematological malignancies	AZD1390 ATM glioblastoma	AZD0171 + <i>Imfinzi</i> + CTx anti-LIF+PD-L1+CTx 1L metastatic pancreatic ductal adenocarcinoma	AZD4573 CDK9 haematological malignancies	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
AZD2936 ARTEMIDE-1# PD1/TIGIT bispecific mAb solid tumours	AZD5991 MCL1 haematological malignancies	AZD4573 + <i>Calquence</i> CDK9+BTK haematological malignancies	AZD5305 PARP1Sel solid tumours	capivasertib + abiraterone CAPitello-281# AKT+abiraterone PTEN deficient mHSPC	capivasertib + CTx CAPitello-290# AKT+chemotherapy mTNBC 1L
AZD7789 PD1/TIM3 bispecific mAb solid tumours, haem malignancies	AZD8205 B7-H4 TOP1i ADC solid tumours	camizestrant SERD ER+ breast	capivasertib# AKT inhibitor prostate cancer	capivasertib + docetaxel CAPitello-280# AKT inhibitor + docetaxel	capivasertib + fulvestrant + palbociclib CAPitello-292# AKT+fulvestrant+CDK4/6 HR+ breast 1L
AZD8701 +/- <i>Imfinzi</i> # FOXP3 +/- PD-L1 solid tumours	AZD8853 GDF-15 solid tumours	ceralasertib ATR solid tumours	<i>Imfinzi</i> (platform) COAST# PD-L1+multiple novel ONC therapies NSCLC	capivasertib + fulvestrant CAPitello-291# AKT+fulvestrant locally-advanced (inoperable) or metastatic breast	datopotamab deruxtecan TROPION Lung08# TROP2 ADC 1L metastatic NSCLC
MEDI1191# IL12 mRNA solid tumours	IPH5201# CD39 solid tumours	<i>Imfinzi</i> (platform) HUDSON PD-L1+multiple novel ONC therapies post IO NSCLC	<i>Imfinzi</i> (platform) NeoCOAST# PD-L1+multiple novel ONC therapies NSCLC	datopotamab deruxtecan TROPION-Breast01# TROP2 ADC 2-3L HR+ HER2- BC	datopotamab deruxtecan TROPION-Breast02# TROP2 ADC 1L TNBC
MEDI9253 rNDV IL12 solid tumours	MEDI5752 + lenvatinib PD-1/CTLA-4+VEGF advanced renal cell carcinoma	<i>Imfinzi</i> + <i>Lynparza</i> ORION# PD-L1+PARP 1L mNSCLC	<i>Imfinzi</i> + monalizumab# PD-L1+NKG2A solid tumours	datopotamab deruxtecan TROPION-Lung01# TROP2 ADC 2L+ NSCLC with and without actionable alterations	<i>Imfinzi</i> +- oleclumab +- mona unresectable stage III NSCLC PACIFIC-9# PD-L1+NKG2A or PD-L1+CD73 NSCLC
	<i>Tagrisso</i> + (<i>Koselugo</i> or <i>Orpathys</i>) TATTON# EGFR+MEK/MET advanced EGFRm NSCLC	MEDI5752 PD-1/CTLA-4 solid tumours	oleclumab + CTx or <i>Imfinzi</i> + oleclumab + CTx CD73+chemo or PD-L1+CD73+chemo pancreatic	<i>Imfinzi</i> + tremelimumab + SoC NILE PD-L1+CTLA-4+SoC 1L urothelial cancer	<i>Imfinzi</i> + treme + TACE +/- lenvatinib EMERALD-3 PD-L1+CTLA4+VEGF+/-chemo-embolization LR HCC
			<i>Tagrisso</i> + <i>Orpathys</i> SAVANNAH# EGFR+MET advanced EGFRm NSCLC	<i>Lynparza</i> + <i>Imfinzi</i> + bevacizumab DUO-O# PARP+PD-L1+VEGF 1L ovarian	<i>Imfinzi</i> +/- tremelimumab + CRT ADRIATIC# PD-L1+/-CTLA-4+CRT LS-SCLC
				monalizumab + cetuximab INTERLINK-1# NKG2A+EGFR 2L+ relapsed metastatic HNSCC	<i>Lynparza</i> + <i>Imfinzi</i> DUO-E# PARP+PD-L1 1L endometrial cancer
					<i>Orpathys</i> + <i>Imfinzi</i> SAMETA# MET+PD-L1 1L papillary renal cell carcinoma

Under Review

2 New Molecular Entities

<i>Imfinzi</i> + tremelimumab HIMALAYA# PD-L1+CTLA-4 1L HCC	<i>Imfinzi</i> +/- tremelimumab + CTx POSEIDON PD-L1+/-CTLA-4+CTx 1L NSCLC
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Phase progressions based on first patient dose achievement.

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

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

● Precision medicine approach being explored



Q2 2022 Oncology life-cycle management¹ pipeline

Phase 1 1 Project	Phase 2 7 Projects	Phase 3 30 Projects	Under Review 4 Projects
<i>Enhertu</i> (platform) DESTINY-Breast08# HER2 ADC breast	<i>Enhertu</i> (platform) DESTINY-Breast07# HER2 ADC breast	<i>Calquence</i> + R-CHOP ESCALADE BTK+R-CHOP 1L DLBCL	<i>Calquence</i> ECHO# BTK inhibitor 1L MCL
<i>Enhertu</i> DESTINY-PanTumour01# HER2 targeting ADC HER2 mutant tumours	<i>Enhertu</i> DESTINY-Breast05# HER2 ADC breast	<i>Enhertu</i> DESTINY-Breast02# HER2 ADC breast	<i>Enhertu</i> DESTINY-Breast06# HER2 ADC breast
<i>Enhertu</i> DESTINY-PanTumour02# HER2 targeting ADC HER2-expressing solid tumours	<i>Enhertu</i> DESTINY-Breast09# HER2 ADC breast	<i>Enhertu</i> DESTINY-Breast11# HER2 ADC breast	<i>Enhertu</i> DESTINY-Gastric04# HER2 ADC HER2+ gastric cancer 2L
<i>Imfinzi</i> (platform) BEGONIA PD-L1 1L mTNBC	<i>Imfinzi</i> PEARL PD-L1 1L metastatic NSCLC	<i>Enhertu</i> DESTINY-Lung04# HER2 ADC NSCLC	<i>Imfinzi</i> + CRT KUNLUN# PD-L1+CRT locally-advanced ESCC
<i>Imfinzi</i> (platform) MAGELLAN# PD-L1 1L mNSCLC	<i>Imfinzi</i> + CRT PACIFIC-2# PD-L1+CRT locally-advanced stage III NSCLC	<i>Imfinzi</i> + CRT PACIFIC-5 (China)# PD-L1+CRT locally-advanced stage III NSCLC	<i>Imfinzi</i> + CTx MERMAID-1 PD-L1 stage II-III adjuvant NSCLC
<i>Lynparza</i> (basket) LYNK002# PARP inhibitor HRRm cancer	<i>Imfinzi</i> + CTx NIAGARA PD-L1 mAb + CTx muscle invasive bladder cancer	<i>Imfinzi</i> + CTx neoadjuvant AEGEAN# PD-L1+CTx locally-advanced stage II-III NSCLC	<i>Imfinzi</i> + domvanalimab (AB154) + CTx PACIFIC-8# PD-L1+TIGIT+CTx unresectable stage III NSCLC
<i>Tagrisso</i> ORCHARD platform study# EGFR+multiple novel ONC therapies EGFRm NSCLC	<i>Imfinzi</i> + EV +/- treme VOLGA PD-L1 + nectin-4 targeting ADC +/- CTLA4 muscle invasive bladder cancer	<i>Imfinzi</i> + FLOT MATTERHORN# PD-L1+CTx neo-adjuvant/adjuvant gastric cancer	<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> POTOMAC PD-L1 mAb non-muscle invasive bladder cancer	<i>Imfinzi</i> MERMAID-2 PD-L1 stage II-III premetastatic NSCLC
	<i>Imfinzi</i> post-SBRT PACIFIC-4# PD-L1 post-SBRT stage I/II NSCLC	<i>Lynparza</i> MONO-OLA1# PARP 1L BRCAwt ovarian cancer	<i>Tagrisso</i> +/- CTx neoadjuvant NeoADAURA EGFR inhibitor +/- CTx stage II/III resectable EGFRm NSCLC
	<i>Tagrisso</i> + CTx FLAURA2 EGFR+chemo 1L adv EGFRm NSCLC	<i>Tagrisso</i> LAURA EGFR inhibitor stage III EGFRm non-small cell lung cancer	<i>Tagrisso</i> ADAURA2 EGFR inhibitor adjuvant EGFRm NSCLC stage Ia2-Ia3 following complete tumour resection

Phase progressions based on first patient dose achievement.

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

Partnered and/or in collaboration; [†]Registrational Phase II/III trial



Q2 2022 BioPharmaceuticals new molecular entity¹ pipeline

Phase 1 15 New Molecular Entities		Phase 2 17 New Molecular Entities		Phase 3 4 New Molecular Entities	Under Review 2 New Molecular Entities
AZD0780 PCSK9 dyslipidemia	AZD2373 Podocyte health nephropathy	AZD1402# Inhaled IL-4Ra asthma	AZD4831 MPO HFpEF	brazikumab INTREPID IL-23 mAb Crohn's disease	nirsevimab# RSV mAb-YTE passive RSV immunisation
AZD2693 nonalcoholic steatohepatitis	AZD3366 CD39L3 CV disease	AZD5718 FLAP coronary artery disease / CKD	AZD5718 FLAP inhibition asthma	eplontersen# LICA ATTR-Cardiomyopathy	PT027# ICS/SABA asthma
AZD3427 Relaxin mimetic CV disease	AZD4041# orexin 1 receptor antagonist opioid use disorder	AZD7986# DPP1 COPD	AZD8233 PCSK9-ASO hypercholesterolemia	eplontersen# LICA hATTR-Polyneuropathy	
AZD4604 inhaled JAK1 asthma	AZD5055 Porcupine inhibitor IPF	AZD9977 + <i>Farxiga/Forxiga</i> MR+SGLT2 HF with CKD	cotadutide GLP-1/glucagon T2D / obesity / NASH / DKD	tozorakimab IL-33 mAb COPD	
AZD5462 Relaxin mimetic CV disease	AZD7503 ASO NASH	brazikumab EXPEDITION IL-23 mAb ulcerative colitis	MEDI7352 NGF/TNF OA pain / painful diabetic neuropathy		
AZD8630# Inhaled TSLP FAb asthma	MEDI0618 PAR2 antagonist mAb osteoarthritis pain	MEDI6570 LOX-1 CV disease	tozorakimab IL-33 mAb diabetic kidney disease		
MEDI1341# alpha synuclein mAb parkinson's disease	MEDI1814# amyloid beta mAb alzheimer's disease	navafenterol# MABA chronic obstructive pulmonary disease	tozorakimab IL-33 mAb COVID-19		
MEDI8367 avb8 chronic kidney disease		tozorakimab IL-33 mAb asthma	zibotentan + <i>Farxiga/Forxiga</i> ETA antagonist+SGLT2 CKD		
		tozorakimab IL-33 mAb atopic dermatitis			

Phase progressions based on first patient dose achievement.

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

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

● Precision medicine approach being explored



Q2 2022 BioPharmaceuticals life-cycle management¹ pipeline

Phase 1 0 Projects	Phase 2 4 Projects	Phase 3 17 Projects	Under Review 0 Projects
	<i>Andexxa</i> (ALXN2070) anti-factor Xa reversal urgent surgery	<i>Breztri/Triexo</i> (PT010) KALOS LOGOS LABA/LAMA/ICS asthma	<i>Farxiga/Forxiga</i> DAPA-MI SGLT2 prevention of HF and CV death following a myocardial infarction
	<i>Fasenra</i> ARROYO IL-5R mAb chronic spontaneous urticaria	<i>Farxiga/Forxiga</i> DELIVER SGLT2 HFpEF	<i>Fasenra</i> FJORD IL-5R mAb bullous pemphigoid
	roxadustat# HIFPH anaemia CIA	<i>Fasenra</i> HUDSON IL-5R mAb eosinophilic gastritis and eosinophilic gastroenteritis	<i>Fasenra</i> MAHALE IL-5R mAb non-cystic fibrosis bronchiectasis
	<i>Tezspire</i> COURSE# TSLP mAb chronic obstructive pulmonary disease	<i>Fasenra</i> MANDARA IL-5R EGPA	<i>Fasenra</i> MESSINA IL-5R mAb eosinophilic oesophagitis
		<i>Fasenra</i> NATRON IL-5R mAb hypereosinophilic syndrome	<i>Fasenra</i> ORCHID# IL-5R mAb nasal polyps
		<i>Fasenra</i> RESOLUTE# IL-5R COPD	<i>Lokelma</i> DIALIZE-Outcomes potassium binder CV outcomes in patients on chronic haemodialysis with hyperkalaemia
		<i>Lokelma</i> STABILIZE-CKD potassium binder hyperkalaemia in CKD	roxadustat# HIFPH anaemia MDS
		<i>Saphnelo</i> TULIP-SC# Type I IFN receptor SLE SC	<i>Saphnelo</i> # Type I IFN receptor mAb lupus nephritis
		<i>Tezspire</i> WAYPOINT# TSLP mAb nasal polyps	

Phase progressions based on first patient dose achievement.

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

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

● Precision medicine approach being explored



Q2 2022 Rare Disease pipeline¹

Phase 1 5 Projects	Phase 2 4 Projects	Phase 3 7 Projects	Under Review 1 Project
ALXN1720 anti-C5 bi-specific minibody generalised myasthenia gravis	ALXN2050 oral factor D inhibitor paroxysmal nocturnal haemoglobinuria	acoramidis (ALXN2060)# oral TTR stabilizer transthyretin amyloid cardiomyopathy	<i>Ultomiris</i> (ALXN1210) anti-complement C5 mAb subcutaneous, paroxysmal nocturnal haemoglobinuria and atypical haemolytic uraemic syndrome
ALXN1820 anti-properdin bi-specific haematology	ALXN2050 oral factor D inhibitor generalised myasthenia gravis	ALXN1840 bis-choline tetrathiomolybdate Wilson disease	
ALXN1850 next gen TNSALP ERT hypophosphatasia	danicopan (ALXN2040) factor D inhibitor geographic atrophy	CAEL-101 fibril-reactive mAb AL amyloidosis	
ALXN1910 next gen TNSALP ERT bone metabolism	<i>Ultomiris</i> (ALXN1210) anti-complement C5 mAb dermatomyositis	danicopan (ALXN2040) factor D inhibitor paroxysmal nocturnal haemoglobinuria with extravascular haemolysis	
NI006# TTR depleter transthyretin amyloid cardiomyopathy		<i>Ultomiris</i> (ALXN1210) anti-complement C5 mAb neuromyelitis optica spectrum disorder	
		<i>Ultomiris</i> (ALXN1210) anti-complement C5 mAb haematopoietic stem cell transplant-associated thrombotic microangiopathy	
		<i>Ultomiris</i> (ALXN1210) anti-complement C5 mAb complement-mediated thrombotic microangiopathy	

Phase progressions based on first patient dose achievement.

¹ Includes new molecular entities and significant life-cycle management projects

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

● Precision medicine approach being explored



Estimated key regulatory submission acceptances

NME	H2 2022			H1 2023			H2 2023			>2023		
LCM	eplontersen Polyneuropathy	datopotamab deruxtecan TROPION-Lung01 2L+ NSCLC with and without actionable alterations	capivasertib + fulvestrant CAPITello-291 locally-advanced (inoperable) mBC	capivasertib + CTx CAPITello-290 mTNBC 1L	capivasertib + fulvestrant + palbociclib CAPITello-292 HR+ breast 1L	capivasertib + abiraterone CAPITello-281 PTEN deficient mHSPC	capivasertib + docetaxel CAPITello-280 prostate cancer	datopotamab deruxtecan TROPION Lung08 1L metastatic NSCLC	ALXN1840 Wilson's disease			
	H2 2022	H1 2023	H2 2023									
	Enhertu DESTINY-Breast02 ADC breast	Imfinzi PEARL metastatic NSCLC	Imfinzi + CTx neoadjuvant AEGEAN locally-advanced stage II-III NSCLC	Calquence + R-CHOP ESCALADE BTK+R-CHOP 1L DLBCL	Imfinzi post-SBRT PACIFIC-4 post-SBRT stage I/II NSCLC	Fasenra RESOLUTE COPD						
	Imfinzi + VEGF + TACE EMERALD-1 locoregional HCC	Imfinzi + CRT PACIFIC-5 (China) locally-advanced stage III NSCLC	Imfinzi + CTx NIAGARA muscle invasive bladder cancer	Calquence + venetoclax + obinutuzumab AMPLIFY 1L CLL	Imfinzi MERMAID-2 stage II-III premetastatic NSCLC	Fasenra FJORD bullous pemphigoid						
Farxiga/Forxiga DELIVER HFpEF	Imfinzi + CRT PACIFIC-2 locally-advanced stage III NSCLC	Imfinzi + CTx TOPAZ-1 biliary tract cancer	Calquence ECHO 1L MCL	Imfinzi POTOMAC non-muscle invasive bladder cancer	Fasenra MAHALE non-cystic fibrosis bronchiectasis							
Ultomiris (ALXN1210) NMOSD	Imfinzi + VEGF EMERALD-2 adjuvant HCC	Tagrisso LAURA stage III EGFRm NSCLC	Enhertu DESTINY-Breast11 ADC breast	Lynparza MONO-OLA1 1L BRCAwt ovarian cancer	Fasenra MANDARA EGPA							
	Fasenra MESSINA eosinophilic oesophagitis	Tagrisso + CTx FLAURA2 1L adv EGFRm NSCLC	Enhertu DESTINY-Breast05 ADC breast	Imfinzi + domvanalimab (AB154) + CTx PACIFIC-8 unresectable stage III NSCLC	Fasenra NATRON hypereosinophilic syndrome							
			Enhertu DESTINY-Breast06 ADC breast	Tagrisso ADAURA2 adjuvant EGFRm NSCLC	Saphnelo TULIP-SC SLE SC							
			Enhertu DESTINY-Breast09 ADC breast	Tagrisso +/- CTx neoadjuvant NeoADAURA stage II/III resectable EGFRm NSCLC	Tezspire WAYPOINT nasal polyps							
			Enhertu DESTINY-Gastric04 gastric cancer 2L	Farxiga/Forxiga DAPA-MI prevention of HF and CV death following a myocardial infarction	Fasenra ORCHID nasal polyps							
			Enhertu DESTINY-Lung04 ADC NSCLC	Lokelma DIALIZE-Outcomes	Fasenra HUDSON EG/EGE							
			Imfinzi + CRT KUNLUN locally-advanced ESCC	Lokelma STABILIZE-CKD	Ultomiris dermatomyositis							
			Imfinzi + CTx MERMAID-1 stage II-III adjuvant NSCLC	roxadustat anaemia MDS	Ultomiris anti-complement C5 mAb haematopoietic STC-associated TMA							
			Imfinzi + EV +/- treme VOLGA muscle invasive bladder cancer	Saphnelo lupus nephritis	Ultomiris complement-mediated TMA							
			Imfinzi + FLOT MATTERHORN neo-adjuvant/adjuvant gastric cancer	Breztri/Trixexo (PT010) KALOS LOGOS asthma								



Designations in our pipeline

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Accelerated approvals

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

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Breakthrough / PRIME¹ / Sakigake²

Calquence CLL ELEVATE-TN, ASCEND (US)
Calquence MCL 1L (US)
danicopan (ALXN2040) PNH-EVH (US)
danicopan (ALXN2040) PNH-EVH (EU)
Enhertu HER2+ breast 2L DESTINY-Breast03 (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)
Enhertu HER2-low unresectable and/or metastatic breast cancer DESTINY-Breast04 (US)
Forxiga CKD DAPA-CKD (US)
Koselugo NFI type 1 SPRINT (US)
nirsevimab RSV mAb-YTE MELODY-MEDLEY (US)
nirsevimab RSV mAb-YTE MELODY-MEDLEY (CN)
nirsevimab RSV mAb-YTE MELODY-MEDLEY (EU) ¹
Soliris GBS (JP) ²
Tagrisso adjuvant NSCLC ADAURA (US)
Tezspire asthma NAVIGATOR (US)

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Fast Track

CAEL-101 AL amyloidosis (US)
camizestrant 1L HR+ HER2- ESR1m breast cancer SERENA-6 (US)
cotadutide NASH (US)
Fasenra EG/EGE HUDSON (US)
Forxiga CKD DAPA-CKD (US)
Forxiga MI RRCT DAPA-MI (US)
Lokelma ESRD DIALIZE-OUTCOMES (US)
nirsevimab RSV mAb-YTE MELODY-MEDLEY (US)
Saphnelo SLE (US)

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Priority Review

Calquence MCL 1L (US)
Enhertu HER2+ breast 2L DESTINY-Breast03 (US)
Enhertu HER2+ breast 2L DESTINY-Breast03 (CN)
Enhertu HER2-low unresectable and/or metastatic breast cancer DESTINY-Breast04 (US)
Enhertu HER2+/HER2-low gastric 3L DESTINY-Gastric01 (US)
Enhertu HER2mut NSCLC 2L+ DESTINY-Lung01 (US)
Forxiga CKD DAPA-CKD (US)
Forxiga CKD DAPA-CKD (JP)
Imfinzi + treme HCC 1L (HIMALAYA) (US)
Imfinzi + CTx BTC 1L (TOPAZ-1) (US)
Koselugo NFI type 1 SPRINT (US)
Lynparza gBRCA adj breast OlympiA (US)
Roxadustat chronic kidney disease (CN)
Tagrisso adjuvant NSCLC (ADAURA) (US)
Tezspire asthma NAVIGATOR (US)
Ultomiris gMG (US)

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Orphan

ALXN1840 WD (US)
ALXN1840 WD (EU)
ALXN2050 PNH (US)
ALXN2050 PNH (EU)
Andexxa Acute Major Bleed (JP)
CAEL-101 AL amyloidosis (US)
CAEL-101 AL amyloidosis (EU)
Calquence CLL 1L (US)
Calquence CLL 1L (EU)
Calquence MCL 1L (US)
danicopan (ALXN2040) PNH (US)
danicopan (ALXN2040) PNH (EU)
Enhertu HER2+/HER2-low gastric 3L DESTINY-Gastric01 (US)
eplontersen transthyretin-mediated amyloidosis (US)
Fasenra EG/EGE HUDSON (US)
Fasenra EGPA MANDARA (US)
Fasenra EOE MESSINA (US)
Fasenra HES NATRON (US)
Imfinzi + CTx Biliary Tract 1L TOPAZ-1 (US)
Imfinzi + CTx Biliary Tract 1L TOPAZ-1 (JP)
Imfinzi +/- treme HCC 1L (EU)
Imfinzi +/- treme HCC 1L (US)
Koselugo NFI type 1 SPRINT (US)
Koselugo NFI type 1 SPRINT (EU)
Koselugo NFI type 1 SPRINT (JP)
Lynparza gBRCA adj breast OlympiA (JP)
Tezspire eosinophilic oesophagitis (US)
Ultomiris DM (US)
Ultomiris HSCT-TMA (US)
Ultomiris SC PNH (US)

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

BREAKTHROUGH DESIGNATION is a process designed to expedite the development and review of medicines which may demonstrate substantial improvement over available therapy. 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.

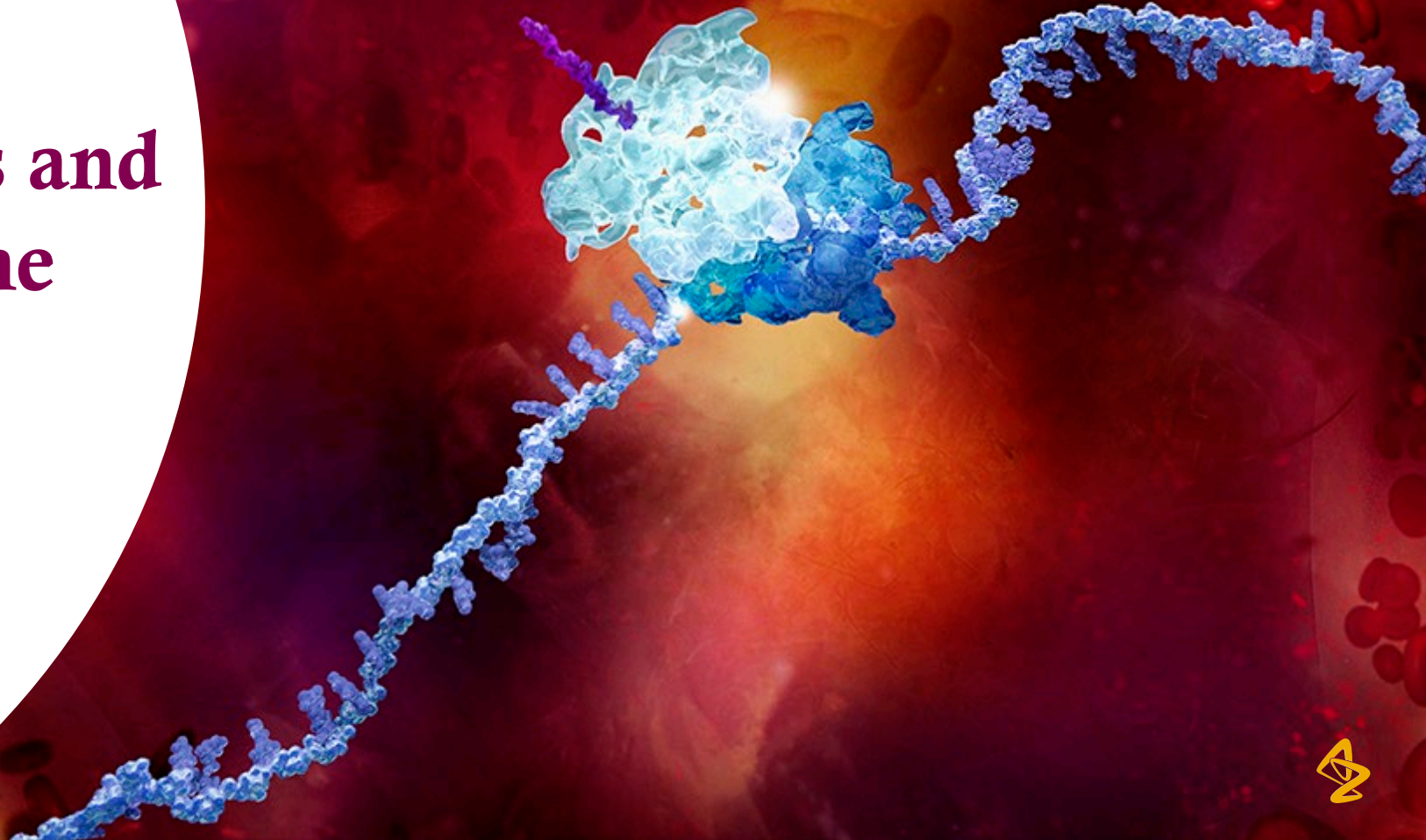
FAST TRACK is a process designed to facilitate the development, and expedite the review of medicines to treat serious conditions and fill an unmet medical need.

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.



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



Tagrisso (highly-selective, irreversible EGFRi)

NSCLC

Trial	Population	Patients	Design	Endpoints	Status
Phase III ADAURA NCT02511106	Adjuvant EGFRm NSCLC	682	<ul style="list-style-type: none"> Arm 1: <i>Tagrisso</i> QD following complete tumour resection, with or without chemo Arm 2: placebo Global trial - 25 countries 	<ul style="list-style-type: none"> Primary endpoint: DFS Secondary endpoints: DFS Rate, OS, OS Rate, QoL 	<ul style="list-style-type: none"> FPCD: Q4 2015 LPCD: Q1 2019 Data readout: Q2 2020 Trial unblinded due to efficacy DFS primary endpoint met
Phase III LAURA NCT03521154	Maintenance therapy in patients with locally advanced, unresectable EGFRm Stage III NSCLC whose disease has not progressed following platinum-based chemoradiation therapy	200	<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, DCR 	<ul style="list-style-type: none"> FPCD: Q4 2018 Data anticipated: H2 2023
Phase III ADAURA2 NCT05120349	Adjuvant EGFRm NSCLC Stage IA2 - 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, QoL 	<ul style="list-style-type: none"> FPCD: Q2 2022 Data anticipated: >2023
Phase II ELIOS NCT03239340	EGFR TKI treatment-naïve patients with locally-advanced or metastatic EGFRm NSCLC	150	<ul style="list-style-type: none"> Single arm trial - <i>Tagrisso</i> Global trial - five countries 	<ul style="list-style-type: none"> Primary endpoint: proportion of patients with a given tumour genetic and proteomic marker at the point of disease progression as defined by the investigator Secondary endpoints: PFS, ORR, DoR 	<ul style="list-style-type: none"> FPCD: Q2 2018
Phase I ODIN-BM NCT03463525	Patients with EGFRm NSCLC with brain metastases	8	<ul style="list-style-type: none"> Single-arm trial - <i>Tagrisso</i> 	<ul style="list-style-type: none"> Primary endpoints: assessments of brain standard uptake value (SUV) and pharmacokinetics (PK) Secondary endpoint: PK 	<ul style="list-style-type: none"> FPCD: Q4 2018 LPCD: Q1 2020 Data readout: Q4 2021



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 plus pemetrexed/carboplatin or pemetrexed/cisplatin Arm 2: <i>Tagrisso</i> plus 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, OS 	<ul style="list-style-type: none"> FPCD: Q1 2021 Data anticipated: >2023
Phase III FLAURA2 NCT04035486	1st-line EGFRm NSCLC	586	<ul style="list-style-type: none"> Arm 1: <i>Tagrisso</i> plus 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, PK 	<ul style="list-style-type: none"> FPCD: Q4 2019 Data anticipated: H1 2023
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> plus pemetrexed/carboplatin or pemetrexed/cisplatin Arm 2: placebo plus pemetrexed/carboplatin or pemetrexed/cisplatin Global trial 	<ul style="list-style-type: none"> Primary endpoint: PFS Secondary endpoints: Intracranial PFS, Extracranial PFS, OS 	<ul style="list-style-type: none"> FPCD: Q3 2021 Data anticipated: >2023
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> Arm2: Pemetrexed with either cisplatin or carboplatin 	<ul style="list-style-type: none"> Primary endpoint: PFS Secondary endpoints: OS, ORR, PK, DCR, DoR 	<ul style="list-style-type: none"> FPCD: Q3 2022 Data anticipated: >2023 Initiating
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: >2023



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: >2023
Phase II SAVANNAH NCT03778229	EGFRm / MET+, locally advanced or metastatic NSCLC who have progressed following treatment with <i>Tagrisso</i>	294	<ul style="list-style-type: none"> Single arm trial: <i>Tagrisso</i> + <i>Orpathys</i> Global trial 	<ul style="list-style-type: none"> Primary endpoint: ORR Secondary endpoints include PFS, DoR and OS 	<ul style="list-style-type: none"> FPCD: Q1 2019 Data readout: Q2 2022
Phase II ORCHARD NCT03944772	Advanced EGFRm NSCLC patients who have progressed on first line <i>Tagrisso</i> treatment	182	<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 - 8 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: >2023
Phase II NCT04606771	EGFRm/MET amplified advanced NSCLC	56	<ul style="list-style-type: none"> <i>Tagrisso</i> and <i>Orpathys</i> contribution of components 	<ul style="list-style-type: none"> Primary endpoint: ORR Secondary endpoints: PFS, DoR, OS 	<ul style="list-style-type: none"> FPCD: Q4 2020 Data anticipated: H2 2022



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 for Arm 2 vs Arm 3 Secondary endpoint: PFS for Arm 1 vs Arm 3, OS 	<ul style="list-style-type: none"> FPCD: Q1 2019 LPCD: Q3 2021 Data anticipated: H2 2022
Phase III EMERALD-2 NCT03847428	Adjuvant therapy in HCC	877	<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 for Arm 1 vs Arm 3 Secondary endpoints: RFS Arm 2 vs Arm 3, OS, RFS at 24m 	<ul style="list-style-type: none"> FPCD: Q2 2019 LPCD: Q2 2022 Data anticipated: H1 2023
Phase III KUNLUN NCT04550260	Locally advanced, unresectable ESCC	600	<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 Data anticipated: >2023
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, pCR Arm 1 vs Arm 2 	<ul style="list-style-type: none"> FPCD: Q4 2020 LPCD: Q3 2022 Data anticipated: >2023
Phase III HIMALAYA NCT03298451	HCC 1L	1324	<ul style="list-style-type: none"> Arm 1: <i>Imfinzi</i> + tremelimumab Arm 2: <i>Imfinzi</i> Arm 3: sorafenib 	<ul style="list-style-type: none"> Primary endpoint: OS Secondary endpoints: PFS, TTP, ORR 	<ul style="list-style-type: none"> FPCD: Q4 2017 LPCD: Q4 2019 Data readout: Q4 2021 Primary endpoint met
Phase III TOPAZ-1 NCT03875235	BTC 1L	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, DoR 	<ul style="list-style-type: none"> FPCD: Q2 2019 LPCD: Q4 2020 Data readout: Q4 2021 Primary endpoint met
Phase III EMERALD-3 NCT05301842	Locoregional Hepatocellular Carcinoma	525	<ul style="list-style-type: none"> Arm A: TACE + T300 + D + Lenva Arm B: TACE + T300 + D Arm C: TACE 	<ul style="list-style-type: none"> Progression Free Survival (PFS) for Arm A vs Arm C Progression Free Survival (PFS) for Arm B vs Arm C Overall Survival (OS) for Arm A vs Arm C Overall Survival (OS) for Arm B vs Arm C 	<ul style="list-style-type: none"> FPCD: Q2 2022 Data anticipated: >2023



Imfinzi (PD-L1 mAb)

Lung cancer

Trial	Population	Patients	Design	Endpoints	Status
Phase III MERMAID-1 NCT04385368	Completely resected Stage II and III NSCLC	86	<ul style="list-style-type: none"> Arm 1: <i>Imfinzi</i> + SoC chemo Arm 2: placebo + SoC chemo 	<ul style="list-style-type: none"> Primary endpoint: DFS Secondary endpoints: DFS, OS 	<ul style="list-style-type: none"> FPCD: Q3 2020 Data anticipated: >2023
Phase III MERMAID-2 NCT04642469	Completely resected Stage II-III NSCLC	20	<ul style="list-style-type: none"> Arm 1: <i>Imfinzi</i> Arm 2: placebo 	<ul style="list-style-type: none"> Primary endpoint: DFS Secondary endpoints: DFS, PFS, OS 	<ul style="list-style-type: none"> FPCD: Q3 2021 Data anticipated: >2023
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 chemo Arm 2: placebo + platinum-based chemo 	<ul style="list-style-type: none"> Primary endpoints: pCR, EFS Secondary endpoint: mPR 	<ul style="list-style-type: none"> FPCD: Q1 2019 Data anticipated: H1 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 12m 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: >2023
Phase III PACIFIC-2 NCT03519971	Unresected, locally-advanced NSCLC	300	<ul style="list-style-type: none"> Arm 1: <i>Imfinzi</i> i.v. Q4W + chemo/RT Arm 2: placebo + chemo/RT ex US global trial 	<ul style="list-style-type: none"> Primary endpoint: PFS Secondary endpoints: OS, ORR 	<ul style="list-style-type: none"> FPCD: Q2 2018 LPCD: Q3 2019 Data anticipated: H2 2022
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: >2023
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 chemo/RT Arm 2: placebo following chemo/RT ex US global trial, 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: H1 2023



Imfinzi (PD-L1 mAb)

Lung cancer

Trial	Population	Patients	Design	Endpoints	Status
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 chemo/RT Arm 2: <i>Imfinzi</i> + PBO following chemo/RT 	<ul style="list-style-type: none"> Primary endpoint: PFS Secondary endpoint: OS 	<ul style="list-style-type: none"> FPCD: Q1 2022 Data anticipated: >2023
Phase III PEARL NCT03003962	NSCLC 1L	650	<ul style="list-style-type: none"> Arm 1: <i>Imfinzi</i> Q4W Arm 2: chemotherapy Asia trial 	<ul style="list-style-type: none"> Primary endpoint: OS 	<ul style="list-style-type: none"> FPCD: Q1 2017 LPCD: Q1 2019 Data anticipated: H2 2022
Phase III POSEIDON NCT03164616	NSCLC 1L	1000	<ul style="list-style-type: none"> Arm 1: <i>Imfinzi</i> + chemo Arm 2: <i>Imfinzi</i> + tremelimumab + chemo Arm 3: SoC 	<ul style="list-style-type: none"> Primary endpoints: OS, PFS 	<ul style="list-style-type: none"> FPCD: Q2 2017 LPCD: Q4 2018 Data readout: Q4 2019 PFS primary endpoint met OS data readout Q2 2021
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> + tremelimumab (4 doses) Arm 2: <i>Imfinzi</i> Arm 3: placebo 	<ul style="list-style-type: none"> Primary endpoints: PFS, OS 	<ul style="list-style-type: none"> FPCD: Q4 2018 Data anticipated: H1 2023
Phase III PACIFIC-9 NCT05221840 Partnered (Innate)	Patients with locally advanced (Stage III), unresectable NSCLC, who have not progressed following platinum-based cCRT	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 endpoint: OS, ORR, DoR, PFS2, TFST 	<ul style="list-style-type: none"> FPCD: Q2 2022 Data anticipated: >2023
Phase II HUDSON NCT03334617	NSCLC, patients who progressed on an anti-PD-1/PD-L1 containing therapy	340	<ul style="list-style-type: none"> Open-label, biomarker-directed, multicentre trial Module 1: <i>Imfinzi</i> and <i>Lynparza</i> Module 2: <i>Imfinzi</i> and danvatirsen Module 3: <i>Imfinzi</i> and ceralasertib (AZD6738) Module 4: <i>Imfinzi</i> and vistusertib Module 5: <i>Imfinzi</i> and oleclumab Module 6: <i>Imfinzi</i> and <i>Enhertu</i> Module 7: <i>Imfinzi</i> and cediranib Module 8: ceralasertib Module 9: <i>Imfinzi</i> and ceralasertib Module 10: <i>Imfinzi</i> and ceralasertib Module 11: Ceralasertib 	<ul style="list-style-type: none"> Primary outcome: ORR Secondary outcomes: efficacy including OS, PFS, DCR, and safety and tolerability, DoR 	<ul style="list-style-type: none"> FPCD: Q1 2018 Data anticipated: >2023



Imfinzi (PD-L1 mAb)

Lung cancer

Trial	Population	Patients	Design	Endpoints	Status
Phase II COAST NCT03822351	Stage III NSCLC unresectable	189	<ul style="list-style-type: none"> Arm A: <i>Imfinzi</i> Arm B: <i>Imfinzi</i> + oleclumab Arm C: <i>Imfinzi</i> + monalizumab 	<ul style="list-style-type: none"> Primary endpoint: OR per RECIST v1.1 	<ul style="list-style-type: none"> FPCD: Q4 2018 Data readout: Q3 2021
Phase II NeoCOAST NCT03794544	Resectable, early-stage NSCLC	84	<ul style="list-style-type: none"> Arm A: <i>Imfinzi</i> Arm B: <i>Imfinzi</i> + oleclumab Arm C: <i>Imfinzi</i> + monalizumab Arm D: <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 Data readout: Q1 2022
Phase II MAGELLAN NCT03819465	NSCLC 1L	212	<ul style="list-style-type: none"> Arm A1: <i>Imfinzi</i> Arm A2: <i>Imfinzi</i> + danvatirsen Arm A3: <i>Imfinzi</i> + oleclumab Arm A4: MEDI5752 Arm A5: AZD2936 Arm B1: <i>Imfinzi</i> + Investigator's choice of chemo Arm B2: <i>Imfinzi</i> + danvatirsen + Investigator's choice of chemo Arm B3: <i>Imfinzi</i> + oleclumab + Investigator's choice of chemo Arm B4: MEDI5752 Arm B5: AZD2936 + chemo 	<ul style="list-style-type: none"> Primary endpoints: safety & tolerability Secondary endpoints: ORR, DoR, PFS, OS, PK, ADA 	<ul style="list-style-type: none"> FPCD: Q1 2019 Data anticipated: >2023
Phase II NeoCOAST-2 NCT05061550	Early stage, resectable NSCLC (Stage II to Stage IIIA)	140	<ul style="list-style-type: none"> Open-label trial Arm 1: <i>Imfinzi</i> + oleclumab + chemotherapy Arm 2: <i>Imfinzi</i> + monalizumab + chemotherapy 	<ul style="list-style-type: none"> Primary endpoints: pCR, safety 	<ul style="list-style-type: none"> FPCD: Q2 2022 Data anticipated: >2023
Phase I/II SCoPe-D1 NCT04870112	NSCLC SCLC	124	<ul style="list-style-type: none"> Open-label, multicentre trial to evaluate the safety, pharmacokinetics, and preliminary efficacy of subcutaneous <i>Imfinzi</i> 	<ul style="list-style-type: none"> Primary endpoints: PK, safety 	<ul style="list-style-type: none"> FPCD: Q4 2021 Data anticipated: >2023



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: >2023
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"> Copriary endpoints: pCR, EFS 	<ul style="list-style-type: none"> FPCD: Q4 2018 LPCD: Q3 2021 Data anticipated: H2 2023
Phase III SAMETA NCT05043090	MET-Driven, unresectable and locally advanced or metastatic papillary renal cell carcinoma	200	<ul style="list-style-type: none"> <i>Orpathys</i> + <i>Imfinzi</i> versus sunitinib and <i>Imfinzi</i> monotherapy 	<ul style="list-style-type: none"> Primary endpoint: PFS Secondary endpoints include OS, ORR, DoR and DCR 	<ul style="list-style-type: none"> FPCD: Q4 2021 Data anticipated: >2023
Phase III NILE NCT03682068	Bladder cancer 1L	1292	<ul style="list-style-type: none"> Arm 1: <i>Imfinzi</i> + tremelimumab + 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: H2 2023
Phase III VOLGA NCT04960709	Muscle invasive bladder cancer ineligible to cisplatin	830	<ul style="list-style-type: none"> Arm 1: <i>Imfinzi</i> + tremelimumab + enfortumab vedotin Arm 2: <i>Imfinzi</i> + enfortumab vedotin Arm 3: SoC cystectomy 	<ul style="list-style-type: none"> Primary endpoints: safety, EFS, pCR Secondary endpoints: OS 	<ul style="list-style-type: none"> FPCD: Q4 2021 Data anticipated: >2023
Phase II BEGONIA NCT03742102	mTNBC 1L	203	<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 Global trial 	<ul style="list-style-type: none"> Primary endpoints: safety and tolerability Secondary endpoints: ORR, PFS, DoR, OS, PK, ADA 	<ul style="list-style-type: none"> FPCD: Q1 2019 Data anticipated: H1 2023
Phase I CLOVER NCT03509012	HNSCC, NSCLC, SCLC	167	<ul style="list-style-type: none"> <i>Imfinzi</i> +/- treme in combination with chemoradiation in advanced solid tumours 	<ul style="list-style-type: none"> Primary endpoint: safety 	<ul style="list-style-type: none"> FPCD: Q2 2018 Data readout Q4 2021



Lynparza (PARP inhibitor)

Imfinzi combinations

Trial	Population	Patients	Design	Endpoints	Status
Phase III DUO-O NCT03737643	Advanced ovarian cancer 1L	1256	<ul style="list-style-type: none"> Non tBRCAm (tumour BRCA) patients Arm 1: bevacizumab Arm 2: bevacizumab + <i>Imfinzi</i> Arm 3: bevacizumab + <i>Imfinzi</i> + <i>Lynparza</i> tBRCAm patients bevacizumab (optional) + <i>Imfinzi</i> + <i>Lynparza</i> Global trial 	<ul style="list-style-type: none"> Primary endpoint: PFS Secondary endpoints: OS, PFS2 	<ul style="list-style-type: none"> FPCD: Q1 2019 Data anticipated: H1 2023
Phase III DUO-E NCT04269200	Advanced and recurrent endometrial cancer 1L	699	<ul style="list-style-type: none"> Arm 1: chemo + <i>Imfinzi</i> placebo followed by <i>Imfinzi</i> placebo and <i>Lynparza</i> placebo Arm 2: chemo + <i>Imfinzi</i> followed by <i>Imfinzi</i> + <i>Lynparza</i> placebo Arm 3: chemo + <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, DoR 	<ul style="list-style-type: none"> FPCD: Q2 2020 Data anticipated: H2 2023
Phase II ORION NCT03775486	Stage IV NSCLC whose disease has not progressed following SoC chemo + <i>Imfinzi</i> maintenance therapy 1L	250	<ul style="list-style-type: none"> Arm 1: <i>Imfinzi</i> + <i>Lynparza</i> Arm 2: <i>Imfinzi</i> + placebo Global trial 	<ul style="list-style-type: none"> Primary endpoint: PFS Secondary endpoints: OS, ORR, DoR, PFS in HRRm, PK, ADA 	<ul style="list-style-type: none"> FPCD: Q1 2019 Data readout: Q2 2021
Phase I/II MEDIOLA NCT02734004	gBRCAm ovarian cancer 2L+ gBRCAm HER2-negative breast cancer 1-3L SCLC 2L+ Gastric cancer 2L+	148	<ul style="list-style-type: none"> Dose until progression Arm 1: <i>Lynparza</i> + <i>Imfinzi</i> Global trial 	<ul style="list-style-type: none"> Primary endpoints: DCR at 12 weeks, safety and tolerability 	<ul style="list-style-type: none"> FPCD: Q2 2016 LPCD: Q2 2017
Phase I/II MEDIOLA NCT02734004	gBRCAm ovarian cancer 2L+ Non-gBRCAm ovarian cancer 2L+ Non-gBRCAm ovarian cancer 2L+	115	<ul style="list-style-type: none"> Dose until progression Arm 1: <i>Lynparza</i> + <i>Imfinzi</i> Arm 2: <i>Lynparza</i> + <i>Imfinzi</i> Arm 3: <i>Lynparza</i> + <i>Imfinzi</i> + bevacizumab Global trial 	<ul style="list-style-type: none"> Primary endpoints: DCR at 12 weeks, ORR, safety and tolerability 	<ul style="list-style-type: none"> FPCD: Q2 2018 LPCD: Q2 2020



Lynparza (PARP inhibitor)

Multiple cancers

Trial	Population	Patients	Design	Endpoints	Status
Phase III OlympiA NCT02032823 Partnered (BIG & NRG Oncology)	BRCAm 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 partnership with Breast International Group and National Cancer Institute/NRG Oncology 	<ul style="list-style-type: none"> Primary endpoint: invasive disease-free survival (iDFS) Secondary endpoint: 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	BRCAwT advanced ovarian cancer 1L maintenance	420	<ul style="list-style-type: none"> Arm 1: <i>Lynparza</i> Arm 2: placebo Global trial, 12 countries 	<ul style="list-style-type: none"> Primary endpoints: PFS (BRCAwT HRD+ve), PFS (BRCAwT) Secondary endpoints: OS, TFST, PFS2 	<ul style="list-style-type: none"> FPCD: Q3 2021 Data anticipated: >2023



Lynparza (PARP inhibitor)

Other combinations

Trial	Population	Patients	Design	Endpoints	Status
Phase III PROpel NCT03732820	Metastatic castration-resistant prostate cancer 1L	904	<ul style="list-style-type: none"> Arm 1: <i>Lynparza</i> + abiraterone Arm 2: placebo + abiraterone Global trial, including China cohort 	<ul style="list-style-type: none"> Primary endpoint: rPFS Secondary endpoints: OS 	<ul style="list-style-type: none"> FPCD: Q4 2018 Data readout: Q3 2021 Primary endpoint met
Phase III LYNK-003 NCT04456699 Partnered (Merck Sharp & Dohme LLC)	Advanced colorectal cancer 1L maintenance	525	<ul style="list-style-type: none"> Arm 1: bevacizumab + 5-FU maintenance Arm 2: bevacizumab + <i>Lynparza</i> maintenance Arm 3: <i>Lynparza</i> maintenance Global trial 	<ul style="list-style-type: none"> Primary endpoint: PFS Secondary endpoints: OS, ORR, DoR, AEs 	<ul style="list-style-type: none"> FPCD: Q3 2020 Data readout: Q2 2022 Trial stopped for lack of efficacy
Phase II/III COCOS (GY005) NCT02502266 Partnered (National Cancer Institute)	Recurrent platinum resistant/refractory ovarian cancer	680	<ul style="list-style-type: none"> Arm 1: chemo Arm 2: cediranib + <i>Lynparza</i> Arm 3: cediranib Arm 4: <i>Lynparza</i> US, Canada 	<ul style="list-style-type: none"> Primary endpoints: PFS, OS Secondary endpoints: ORR, QoL, safety 	<ul style="list-style-type: none"> FPCD: Q2 2016 Data anticipated: H1 2023
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 endpoints: ORR Secondary endpoints: DOR, OS, PFS, AE, 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 standard of care HER2 therapies, including trastuzumab emtansine	600	<ul style="list-style-type: none"> Randomised open label parallel assignment Enhertu Physician's choice of lapatinib + capecitabine or trastuzumab + capecitabine 	<ul style="list-style-type: none"> Primacy endpoint: PFS Secondary endpoints: OS, ORR, DoR, CBR 	<ul style="list-style-type: none"> FPCD: Q3 2018 LPCD: Q4 2020 Data anticipated: H2 2022
Phase III DESTINY-Breast03 NCT03529110 Partnered (Daiichi Sankyo)	HER2-positive, unresectable and/or metastatic breast cancer previously treated with trastuzumab and taxane	500	<ul style="list-style-type: none"> Randomised open label parallel assignment Enhertu Ado-trastuzumab emtansine 	<ul style="list-style-type: none"> Primary endpoint: PFS Secondary endpoints: OS, ORR, DoR, 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 patients	540	<ul style="list-style-type: none"> Randomised open label parallel assignment Enhertu Physician's choice of SoC chemo (choice of capecitabine, eribulin, gemcitabine, paclitaxel or nab-paclitaxel) 	<ul style="list-style-type: none"> Primary end point: PFS Secondary endpoints: OS, DoR, 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 patients with residual invasive breast cancer following neoadjuvant therapy	1600	<ul style="list-style-type: none"> Randomised open label parallel assignment Enhertu Ado-trastuzumab emtansine 	<ul style="list-style-type: none"> Primary endpoint: IDFS Secondary endpoints: DFS, OS, DRFI, BMFI 	<ul style="list-style-type: none"> FPCD: Q4 2020 Data anticipated: >2023
Phase III DESTINY-Breast06 NCT04494425 Partnered (Daiichi Sankyo)	HER2-Low, HR+ breast cancer patients whose disease has progressed on endocrine therapy in the metastatic setting	850	<ul style="list-style-type: none"> Randomised open label parallel assignment Enhertu Investigator's choice standard of care chemotherapy (capecitabine, paclitaxel, nab-paclitaxel) 	<ul style="list-style-type: none"> Primary endpoint: PFS Secondary endpoints: OS, DoR, ORR 	<ul style="list-style-type: none"> FPCD: Q3 2020 Data anticipated: H2 2023
Phase III DESTINY-Breast09 NCT04784715 Partnered (Daiichi Sankyo)	HER2-positive, metastatic breast cancer, no prior therapy for advanced or metastatic disease	1134	<ul style="list-style-type: none"> Randomised, parallel assignment Enhertu + placebo Enhertu + pertuzumab Standard of care 	<ul style="list-style-type: none"> Primary endpoint: PFS Secondary endpoints: OS, DoR, ORR 	<ul style="list-style-type: none"> FPCD: Q2 2021 Data anticipated: >2023



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	624	<ul style="list-style-type: none"> Randomized open label parallel assignment Enhertu Enhertu, followed by THP doxorubicin and cyclophosphamide, followed by THP 	<ul style="list-style-type: none"> Primary endpoint: pCR Secondary endpoints: EFS, IDFS, OS 	<ul style="list-style-type: none"> FPCD: Q4 2021 Data anticipated: >2023
Phase Ib/II DESTINY-Breast07 NCT04538742 Partnered (Daiichi Sankyo)	HER2-positive metastatic breast cancer	450	<ul style="list-style-type: none"> Randomised open label sequential assignment Enhertu Enhertu + Imfinzi Enhertu + pertuzumab Enhertu + paclitaxel Enhertu + Imfinzi + paclitaxel Enhertu + tucatinib 	<ul style="list-style-type: none"> Primary endpoints: AE, SAE Secondary endpoints: ORR, PFS, DoR, OS 	<ul style="list-style-type: none"> FPCD: Q1 2021 Data anticipated: >2023
Phase Ib DESTINY-Breast08 NCT04556773 Partnered (Daiichi Sankyo)	HER2-low metastatic breast cancer	185	<ul style="list-style-type: none"> Non-randomised open label parallel assignment Enhertu + capecitabine Enhertu + Imfinzi + paclitaxel Enhertu + capivasertib Enhertu + anastrozole Enhertu + Faslodex 	<ul style="list-style-type: none"> Primary endpoints: AE, SAE Secondary endpoints: ORR, PFS, DoR, 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 Enhertu SoC chemo 	<ul style="list-style-type: none"> Primary endpoint: OS Secondary endpoints: ORR, DoR, PFS, DcR, safety 	<ul style="list-style-type: none"> FPCD: Q2 2021 Data anticipated: >2023
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 Enhertu SoC chemo 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, range of PK endpoints 	<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 Enhertu Western population 	<ul style="list-style-type: none"> Primary endpoint: ORR Secondary endpoints: PFS, ORR, OS, 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	75	<ul style="list-style-type: none"> Open label single group assignment Enhertu China 	<ul style="list-style-type: none"> Primary endpoint: ORR Secondary endpoints: PFS, ORR, DCR, OS, DoR, safety 	<ul style="list-style-type: none"> FPCD: Q2 2021 Data anticipated: H1 2023
Phase Ib/II DESTINY-Gastric03 NCT04379596 Partnered (Daiichi Sankyo)	HER2-overexpressing gastric or gastroesophageal junction cancer patients	255	<ul style="list-style-type: none"> Open label parallel assignment Part 1: To determine recommended Phase II combination dose 5 Arms combine <i>Enhertu</i> with standard of care chemotherapies (5-FU, capecitabine, oxaliplatin) and / or durvalumab Part 2: To assess efficacy of the selected combinations Arm 2A: Standard chemotherapy (control) Arm 2B: <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 	<ul style="list-style-type: none"> Part 1 Primary endpoint: safety Part 2 Primary endpoint: ORR Secondary endpoints: DoR, DCR, PFS, OS, range of PK endpoints, presence of ADAs 	<ul style="list-style-type: none"> FPCD: Q2 2020 Data anticipated: H2 2023



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 Treatment (platinum, pemetrexed and pembrolizumab) 	<ul style="list-style-type: none"> Primary endpoint: PFS Secondary endpoints: OS, CNS-PFS, PFS (INV), ORR, DoR, safety; PK, PK/ADA, PRO-Tolerability, PRO-Pulmonary symptoms 	<ul style="list-style-type: none"> FPCD: Q4 2021 Data anticipated: >2023
Phase II DESTINY-Lung01 NCT03505710 Partnered (Daiichi Sankyo)	HER2-over-expressing or mutated, unresectable and/or metastatic NSCLC	170	<ul style="list-style-type: none"> Non randomised parallel group assignment <i>Enhertu</i> 	<ul style="list-style-type: none"> Primary endpoint: ORR Secondary endpoints: DoR, PFS, OS, DCR 	<ul style="list-style-type: none"> FPCD: Q2 2018 Data readout: Q3 2021 Primary endpoint met
Phase II DESTINY-Lung02 NCT04644237 Partnered (Daiichi Sankyo)	HER2-mutated, unresectable and/or metastatic NSCLC	150	<ul style="list-style-type: none"> Randomised parallel group assignment Arm 1: <i>Enhertu</i> 6.4 mg/kg Arm 2: <i>Enhertu</i> 5.4mg/kg 	<ul style="list-style-type: none"> Primary endpoint: ORR Secondary endpoints: DoR, DCR, PFS, OS, PK 	<ul style="list-style-type: none"> FPCD: Q1 2021 Data anticipated: H1 2023
Phase II DESTINY-PanTumour02 NCT04482309 Partnered (Daiichi Sankyo)	HER2 expressing tumours	280	<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, OS 	<ul style="list-style-type: none"> FPCD: Q4 2020 Data anticipated: H1 2023
Phase II DESTINY-PanTumour01 NCT04639219 Partnered (Daiichi Sankyo)	HER2 mutant tumours	100	<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, PK 	<ul style="list-style-type: none"> FPCD: Q1 2021 Data anticipated: H1 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> 6.4 mg/kg Arm 2: <i>Enhertu</i> 5.4mg/kg 	<ul style="list-style-type: none"> Primary endpoint: ORR Secondary endpoint: ORR, PFS, OS, DoR, DCR, range of PK endpoints 	<ul style="list-style-type: none"> FPCD: Q1 2021 Data anticipated: H1 2023



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	136	<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 standard of care 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 Arm 1: <i>Enhertu</i> + cisplatin + <i>Imfinzi</i> Arm 2: <i>Enhertu</i> + carboplatin + <i>Imfinzi</i> Arm 3: <i>Enhertu</i> + pemetrexed + <i>Imfinzi</i> Arm 4: <i>Enhertu</i> + <i>Imfinzi</i> 	<ul style="list-style-type: none"> Primary endpoint: safety Secondary endpoints: ORR, DoR, DCR, PFS, OS, range of PK endpoints 	<ul style="list-style-type: none"> FPCD: Q4 2021 Data anticipated: >2023
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, ORR Secondary endpoints: DoR, DCR, PFS, TTR, OS 	<ul style="list-style-type: none"> FPCD: Q2 2020
Phase Ib U105 NCT03523572 Partnered (Daiichi Sankyo)	HER2-expressing breast and urothelial cancer	99	<ul style="list-style-type: none"> Non randomised sequential assignment <i>Enhertu</i> + nivolumab Global trial 7 countries 	<ul style="list-style-type: none"> Primary endpoints: DLT, ORR, TEAEs Secondary endpoints: DoR, DCR, PFS, TTR, OS, ORR (investigator) 	<ul style="list-style-type: none"> FPCD: Q3 2018 Data readout: Q3 2021



Calquence (BTK inhibitor)

Blood cancers

Trial	Population	Patients	Design	Endpoints	Status
Phase III ACE-CL-007 (ELEVATE-TN) NCT02475681	Previously untreated CLL	535	<ul style="list-style-type: none"> Arm A: chlorambucil + obinutuzumab Arm B: <i>Calquence</i> + obinutuzumab Arm C: <i>Calquence</i> 	<ul style="list-style-type: none"> Primary endpoint: PFS (Arm A vs. Arm B) Secondary endpoints: IRC (independent review committee) assessed ORR, OS (Arm A vs. Arm B vs. Arm C) 	<ul style="list-style-type: none"> FPCD: Q2 2015 Data readout: Q2 2019 Primary endpoint met
Phase III ACE-CL-311 NCT03836261	Previously untreated CLL fit	981	<ul style="list-style-type: none"> Arm A: <i>Calquence</i> + venetoclax Arm B: <i>Calquence</i> + venetoclax + obinutuzumab Arm C: FCR or BR 	<ul style="list-style-type: none"> Primary endpoint: IRC PFS (A vs C) Secondary endpoint: IRC PFS (B vs C); INV PFS (A vs C; B vs C) 	<ul style="list-style-type: none"> FPCD: Q1 2019 LPCD: Q1 2027 Data anticipated: H2 2023
Phase III ACE-CL-309 (ASCEND) NCT02970318	Relapsed/refractory CLL	306	<ul style="list-style-type: none"> Arm A: <i>Calquence</i> Arm B: rituximab + idelalisib or bendamustine (investigator's choice) 	<ul style="list-style-type: none"> Primary endpoint: IRC assessed PFS (Arm A vs. Arm B) Secondary endpoints: INV-assessed ORR, OS, DoR, PROs 	<ul style="list-style-type: none"> FPCD: Q4 2017 Data readout: Q2 2019 Primary endpoint met
Phase III ACE-CL-006 (ELEVATE-RR) NCT02477696	Relapsed/refractory high risk CLL	533	<ul style="list-style-type: none"> Arm A: <i>Calquence</i> Arm B: ibrutinib 	<ul style="list-style-type: none"> Primary endpoint: PFS Secondary endpoints: comparison of incidence of infections, RTs (Richter's Transformation) and atrial fibrillation, OS 	<ul style="list-style-type: none"> FPCD: Q3 2015 Data readout: Q1 2021 Primary endpoint met
Phase III ACE-LY-308 (ECHO) NCT02972840	Previously untreated MCL	626	<ul style="list-style-type: none"> Arm A: <i>Calquence</i> + bendamustine + rituximab Arm B: 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, OS 	<ul style="list-style-type: none"> FPCD: Q2 2017 LPCD: Q2 2023 Data anticipated: H2 2023
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 endpoints: PFS 	<ul style="list-style-type: none"> FPCD: Q1 2021 Data anticipated: >2023
Phase III D822BC0001 NCT04075292	Untreated Chronic Lymphocytic Leukemia	150	<ul style="list-style-type: none"> Arm A: <i>Calquence</i> Arm B: Chlorambucil + rituximab 	<ul style="list-style-type: none"> Primary endpoint: PFS Secondary endpoint: ORR, DoR 	<ul style="list-style-type: none"> FPCD: Q1 2015 LPCD: Q3 2022 Data anticipated: H2 2023



Calquence (BTK inhibitor)

Blood cancers

Trial	Population	Patients	Design	Endpoints	Status
Phase II 15-H-0016 NCT02337829	Relapsed/refractory and treatment naïve/del17p CLL/SLL	48	<ul style="list-style-type: none"> • <i>Calquence</i> monotherapy • Arm A: lymph node biopsy • Arm B: bone marrow biopsy 	<ul style="list-style-type: none"> • Primary endpoint: ORR 	<ul style="list-style-type: none"> • FPCD: Q4 2014 • Data readout: Q4 2020
Phase Ib ACE-LY-106 NCT02717624	MCL	70	<ul style="list-style-type: none"> • <i>Calquence</i> in combination with bendamustine and rituxumab • Arm A: treatment naïve • Arm B: relapsed/refractory • Arm C: treatment naïve: <i>Calquence</i> + venetoclax + rituxumab 	<ul style="list-style-type: none"> • Primary endpoint: safety 	<ul style="list-style-type: none"> • FPCD: Q2 2016 • LPCD: Q2 2022 • Data anticipated: H2 2022
Phase I/II ACE-CL-001 NCT02029443	CLL/SLL/Richter's transformation	306	<ul style="list-style-type: none"> • <i>Calquence</i> monotherapy • Dose escalation and expansion 	<ul style="list-style-type: none"> • Primary endpoints: safety, PK, PD 	<ul style="list-style-type: none"> • FPCD: Q1 2014 • Data readout: Q4 2021
Phase I/II D8220C0007 NCT03932331	Chinese adults R/R MCL and R/R CLL	105	<ul style="list-style-type: none"> • Part 1: R/R B-cell Malignancies • Part 2: Cohort A: R/R MCL • Part 2: Cohort B: R/R CLL 	<ul style="list-style-type: none"> • Primary endpoints: safety, ORR 	<ul style="list-style-type: none"> • FPCD: Q2 2020 • Data anticipated: >2023 • Initial data readout: MCL: Q4 2021
Phase I ACE-LY-003 NCT02180711	Relapsed/refractory follicular lymphoma	80	<ul style="list-style-type: none"> • Arm A: <i>Calquence</i> • Arm B: <i>Calquence</i> + rituximab • Arm C: <i>Calquence</i> + rituximab + lenalidomide 	<ul style="list-style-type: none"> • Primary endpoint: safety 	<ul style="list-style-type: none"> • FPCD: Q1 2015 • LPCD: Q3 2023 • Data anticipated: >2023
Phase I ACE-CL-003 NCT02296918	CLL/SLL/PLL	69	<ul style="list-style-type: none"> • <i>Calquence</i> + obinutuzumab • Arm A: relapsed/refractory • Arm B: treatment naïve • <i>Calquence</i> + venetoclax + rituxumab • Arm C: relapsed/refractory • Arm D: treatment naïve 	<ul style="list-style-type: none"> • Primary endpoints: safety, ORR • Secondary endpoints: PD, PFS, TTNT, OS 	<ul style="list-style-type: none"> • FPCD: Q4 2014 • Data anticipated: H2 2022



Calquence (BTK inhibitor)

Blood cancers

Approved medicines

Late-stage development

Early development

Trial	Population	Patients	Design	Endpoints	Status
Phase I D8220C00001 NCT03198650	Japanese adults with advanced B-cell malignancies	35	<ul style="list-style-type: none"> Part 1: Dose confirmation in r/r B-cell Malignancies Part 2: Dose expansion in r/r CLL or r/r MCL Calquence + obinutuzumab Part 3: Dose confirmation in treatment naïve CLL 	<ul style="list-style-type: none"> Primary endpoint: safety 	<ul style="list-style-type: none"> FPCD: Q2 2017 Data anticipated: H2 2022
Phase I D8220C00018 NCT04488016	Healthy volunteers	28	<ul style="list-style-type: none"> Part 1: Rel bioavailabilty for capsule vs. tablet Part 2: Rel bioavailabilty for oral solution of tablet 	<ul style="list-style-type: none"> Primary endpoint: safety, bioavailability 	<ul style="list-style-type: none"> FPCD: Q2 2019 Data readout: Q1 2021 Primary endpoint met
Phase I D8223C00013 NCT04768985	Healthy volunteers	66	<ul style="list-style-type: none"> Arm A: Calquence tablet Arm B: Calquence capsule 	<ul style="list-style-type: none"> Primary endpoint: bioequivalence 	<ul style="list-style-type: none"> FPCD: Q1 2021 Data readout: Q4 2021 Primary endpoint met

Oncology

CVRM

R&I

Other

V&I

Rare Disease



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: >2023
Phase II NCT04923932 Partnered (HUTCHMED)	Locally advanced or metastatic gastric cancer and oesophagogastric 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, safety 	<ul style="list-style-type: none"> FPCD: Q3 2021 Data anticipated: >2023



Capivasertib (AKT inhibitor)

Breast cancer, prostate cancer

Trial	Population	Patients	Design	Endpoints	Status
Phase III CAPitello-290 NCT03997123 Partnered (Astex)	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: H2 2023
Phase III CAPitello-291 NCT04305496 Partnered (Astex)	2L and beyond in 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 + Faslodex Arm 2: placebo + Faslodex 	<ul style="list-style-type: none"> Primary endpoint: PFS 	<ul style="list-style-type: none"> FPCD: Q2 2020 Data anticipated: H2 2022
Phase III CAPitello-281 NCT04493853 Partnered (Astex)	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: >2023
Phase III CAPitello-292 NCT04862663 Partnered (Astex)	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 + Faslodex Arm 2: placebo + palbociclib + Faslodex 	<ul style="list-style-type: none"> Primary endpoint: PFS 	<ul style="list-style-type: none"> FPCD: Q2 2021 Data anticipated: >2023
Phase III CAPitello-280 NCT05348577 Partnered (Astex)	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: Overall Survival (OS) 	<ul style="list-style-type: none"> FPCD: Q2 2022 Data anticipated: >2023



Camizestrant (AZD9833, next generation oral SERD)

Breast cancer

Trial	Population	Patients	Design	Endpoints	Status
Phase III SERENA-4 NCT04711252	HR+ HER2- breast cancer	1342	<ul style="list-style-type: none"> Randomised, double-blind, comparative trial Arm A: camizestrant (AZD9833) + palbociclib Arm B: anastrozole + palbociclib 	<ul style="list-style-type: none"> Primary endpoint: PFS Secondary endpoint: OS, PFS2 	<ul style="list-style-type: none"> FPCD: Q1 2021 Data anticipated: >2023
Phase III SERENA-6 NCT04964934	HR+ HER2- breast cancer	300	<ul style="list-style-type: none"> Randomised, double-blind, comparator trial Arm A: camizestrant (AZD9833) plus palbociclib or abemaciclib Arm B: anastrozole or letrozole plus palbociclib or abemaciclib 	<ul style="list-style-type: none"> Primary endpoint: PFS Secondary endpoint: OS, PFS2 	<ul style="list-style-type: none"> FPCD: Q3 2021 Data anticipated: H2 2023
Phase II SERENA-2 NCT04214288	HR+ breast cancer	240	<ul style="list-style-type: none"> Randomised, open-label, parallel-group, multicentre trial camizestrant (AZD9833) vs. i.m. Faslodex in women with advanced breast cancer. 	<ul style="list-style-type: none"> Primary outcome: mPFS 	<ul style="list-style-type: none"> FPCD: Q2 2020 LPCD: Q3 2021 Data anticipated: H1 2023
Phase II SERENA-3 NCT04588298	HR+ breast cancer	84	<ul style="list-style-type: none"> Randomised, open-label, parallel-group, multicentre trial 	<ul style="list-style-type: none"> Primary outcome: change in ER expression between pre- and on-treatment tumour biopsies 	<ul style="list-style-type: none"> FPCD: Q4 2020 Data anticipated: H1 2023
Phase I NCT04541433	HR+ breast cancer	18	<ul style="list-style-type: none"> Open-label anti-tumour activity of camizestrant (AZD9833) in Japanese women with endocrine resistant HR+ HER2- breast cancer that is not amenable to treatment with curative intent. 	<ul style="list-style-type: none"> Primary outcomes: safety and tolerability Secondary outcome: PK 	<ul style="list-style-type: none"> FPCD: Q4 2020 LPCD: Q1 2022 Data anticipated: H1 2023
Phase I SERENA-1 NCT03616587	HR+ breast cancer	305	<ul style="list-style-type: none"> Escalation phase - open label multicentre trial camizestrant (AZD9833) camizestrant (AZD9833) + palbociclib, everolimus, abemaciclib or capivasertib. Expansion phase - randomised expansion cohort(s) camizestrant (AZD9833) camizestrant (AZD9833) + palbociclib, everolimus, abemaciclib or capivasertib. 	<ul style="list-style-type: none"> Primary outcomes: safety and tolerability Secondary outcomes: PK, antitumour activity 	<ul style="list-style-type: none"> FPCD: Q4 2018 Data anticipated: H1 2023



Camizestrant (AZD9833, next generation oral SERD)

Breast cancer

Approved medicines

Late-stage development

Early development

Trial	Population	Patients	Design	Endpoints	Status
Phase I NCT04546347	Healthy volunteers	32	<ul style="list-style-type: none"> Randomised, open-label 	<ul style="list-style-type: none"> Primary outcome: relative bioavailability of different tablet formulations and the effect of food 	<ul style="list-style-type: none"> FPCD: Q3 2020 LPCD: Q4 2020 Data readout: Q3 2021
Phase I NCT04818632	HR+ HER2- breast cancer Chinese patients	30	<ul style="list-style-type: none"> Dose escalation camizestrant (AZD9833) Dose expansion camizestrant (AZD9833) camizestrant (AZD9833) + palbociclib camizestrant (AZD9833) + everolimus 	<ul style="list-style-type: none"> Primary outcomes: safety and tolerability, PK Secondary outcome: antitumour activity 	<ul style="list-style-type: none"> FPCD: Q1 2021 Data anticipated: H2 2023

Oncology

CVRM

R&I

Other

V&I

Rare Disease



Monalizumab (NKG2a mAb)

Cancer

Approved medicines

Late-stage development

Early development

Oncology

Trial	Population	Patients	Design	Endpoints	Status
Phase III INTERLINK-1 NCT04590963 Partnered (Innate Pharma)	Recurrent or metastatic HNSCC, 2L	600	<ul style="list-style-type: none">• Arm A: monalizumab + cetuximab i.v.• Arm B: placebo + cetuximab i.v.• Global	<ul style="list-style-type: none">• Primary: OS• Secondary: PFS, ORR, DoR	<ul style="list-style-type: none">• FPCD: Q4 2020• Data anticipated: >2023

CVRM

R&I

Other

V&I

Rare Disease



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 	<ul style="list-style-type: none"> Primary endpoints: PFS, OS Secondary endpoints: ORR, DoR, TTR, DCR, PK, ADA 	<ul style="list-style-type: none"> FPCD: Q1 2021 Data anticipated: H1 2023
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 	<ul style="list-style-type: none"> Primary endpoints: PFS, OS 	<ul style="list-style-type: none"> FPCD: Q1 2022 Data anticipated: >2023
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, ADA 	<ul style="list-style-type: none"> FPCD: Q1 2021 LPCD: Q1 2022 Data anticipated: >2023
Phase I TROPION-Lung02 NCT04526691 Partnered (Daiichi Sankyo)	Advanced or metastatic NSCLC	120	<ul style="list-style-type: none"> Open label, two-part (dose escalation, dose expansion), sequential assignment datopotamab deruxtecan + pembrolizumab +/- platinum chemotherapy US, Japan 	<ul style="list-style-type: none"> Primary endpoints: DLT, safety Secondary endpoints: ORR, DOR, PFS, OS, PK, ADA 	<ul style="list-style-type: none"> FPCD: Q4 2020 Data anticipated: >2023
Phase I TROPION-Lung04 NCT04612751 Partnered (Daiichi Sankyo)	Advanced or metastatic NSCLC	120	<ul style="list-style-type: none"> Open label, two-part (dose escalation, dose expansion), sequential assignment datopotamab deruxtecan + <i>Imfinzi</i> +/- platinum chemotherapy US, Japan 	<ul style="list-style-type: none"> Primary endpoints: DLT, safety Secondary endpoints: ORR, DOR, PFS, OS, PK, ADA 	<ul style="list-style-type: none"> FPCD: Q1 2021 Data anticipated: >2023



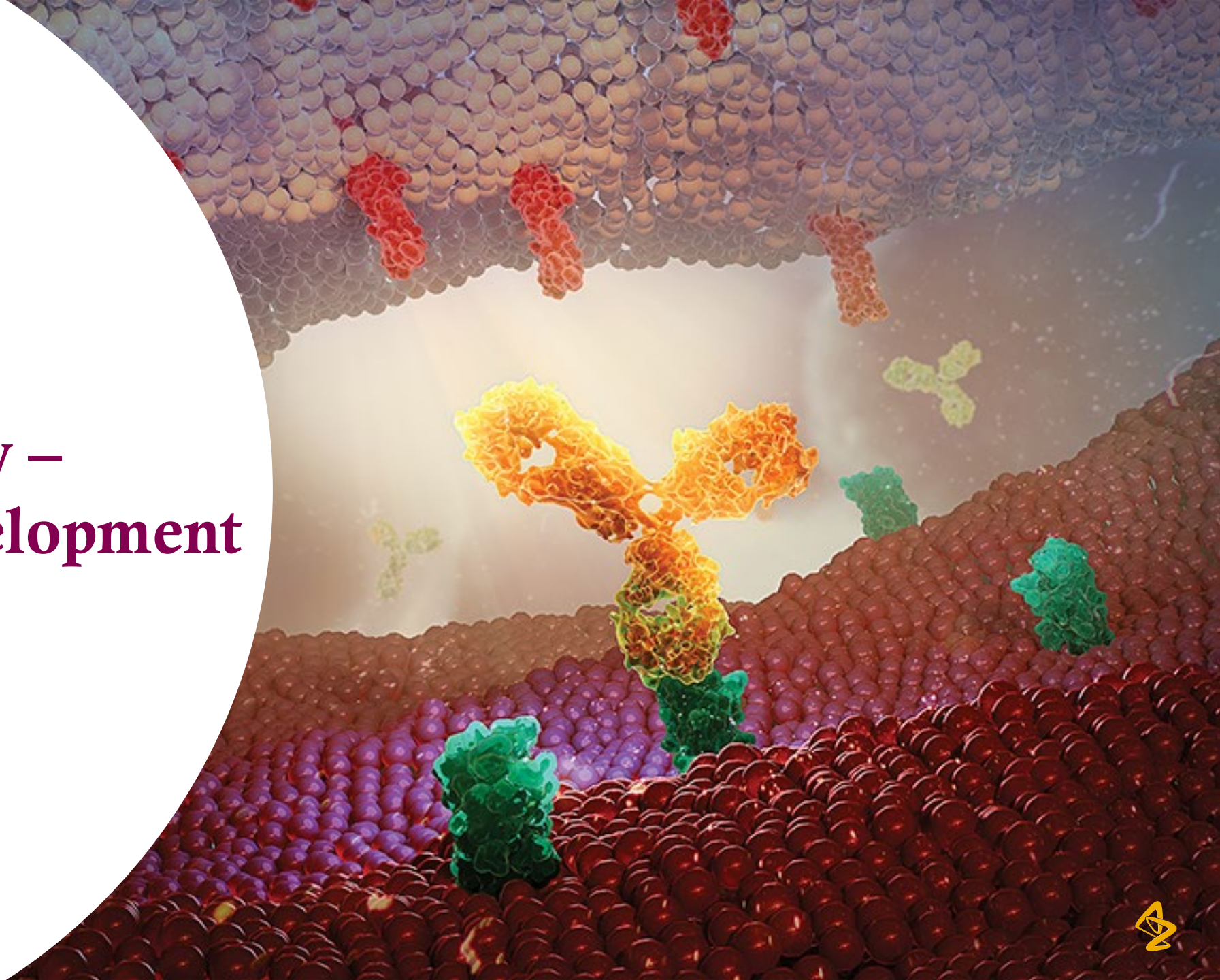
Datopotamab deruxtecan (TROP2 ADC)

NSCLC and other cancers

Trial	Population	Patients	Design	Endpoints	Status
Phase III TROPION-Breast01 NCT05104866 Partnered (Daiichi Sankyo)	Inoperable or metastatic HR+, HER2-breast cancer	700	<ul style="list-style-type: none"> Open-label, randomised datopotamab deruxtecan investigator's choice standard of care chemotherapy (eribulin, vinorelbine, capecitabine, gemcitabine) 	<ul style="list-style-type: none"> Primary endpoints: PFS, OS Secondary endpoints: ORR, DoR, DCR, PK, ADA 	<ul style="list-style-type: none"> FPCD: Q4 2021 Data anticipated: >2023
Phase III TROPION-Breast02 NCT05374512 Partnered (Daiichi Sankyo)	Locally Recurrent Inoperable or Metastatic Triple-negative Breast Cancer	600	<ul style="list-style-type: none"> Open-label, randomised datopotamab deruxtecan global trial investigator's choice of chemotherapy (paclitaxel, nab-paclitaxel, carboplatin, capecitabine, eribulin mesylate) 	<ul style="list-style-type: none"> Primary endpoints: PFS (BICR), OS Secondary endpoints: PFS (Inv), ORR, DoR, PK, ADA 	<ul style="list-style-type: none"> FPCD: Q2 2022 Data anticipated: >2023
Phase I TROPION-PanTumor01 NCT03401385 Partnered (Daiichi Sankyo)	Subjects with advanced solid tumours NSCLC TNBC HR+ BC HER2-negative gastric/GEJ esophageal Urothelial SCLC	770	<ul style="list-style-type: none"> Open label, two-part (dose escalation, dose expansion), sequential assignment datopotamab deruxtecan Japan, US 	<ul style="list-style-type: none"> Primary endpoints: DLT, safety Secondary endpoints: PK, anti-tumour activity, ADA 	<ul style="list-style-type: none"> FPCD: Q1 2018 Data anticipated: >2023 Early data readout (NSCLC) Q1 2021 Early data readout (TNBC) Q2 2021



**Oncology –
early-stage development**



AZD0171 (anti-LIF mAb)

Cancer

Approved medicines

Late-stage development

Early development

Oncology

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 trial of AZD0171 + <i>Imfinzi</i> + gemcitabine, nab-paclitaxel	<ul style="list-style-type: none">Primary endpoints: Safety, OS at 12mSecondary endpoints: ORR, DoR, PFS	<ul style="list-style-type: none">FPCD: Q1 2022Data anticipated: >2023

CVRM

R&I

Other

V&I

Rare Disease



AZD0466 (Bcl2/xL 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/II NCT04865419	Advanced haematologic malignancies	160	<ul style="list-style-type: none"> Module 1 Part A: Dose escalation AZD0466 Part B: Dose expansion AZD0466 Module 2 - DDI trial AZD0466 with voriconazole 	<ul style="list-style-type: none"> Primary endpoint: safety Secondary endpoint: PK 	<ul style="list-style-type: none"> FPCD: Q2 2021 Data anticipated: >2023
Phase I/II NCT05205161	Advanced Non Hodgekin Lymphoma		<ul style="list-style-type: none"> Part A: Dose Escalation Part B: Dose Expansion Arm 1: R/R MCL Part B: Dose Expansion Arm 2: R/R FL or MZL Part B: Dose Expansion Arm 3: R/R DLBCL 	<ul style="list-style-type: none"> Primary endpoints: Part A: Safety Part B: ORR 	<ul style="list-style-type: none"> FPCD: Q3 2022 Data anticipated: >2023



AZD1390 (ATM inhibitor)

Cancer

Approved medicines

Late-stage development

Early development

Oncology

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 A: recurrent GBM, AZD1390 +RT in dose escalation cohortsArm C: primary GBM, AZD1390 +RT in dose escalation cohorts	<ul style="list-style-type: none">Primary endpoints: safety, tolerability, MTDSecondary endpoints: PK and preliminary assessment of anti-tumour activity	<ul style="list-style-type: none">FPCD: Q2 2018Data anticipated: >2023

CVRM

R&I

Other

V&I

Rare Disease



AZD2936 (PD-1/TIGIT Bispecific mAb)

Solid tumours

Trial	Population	Patients	Design	Endpoints	Status
Phase I ARTEMIDE-01 NCT04995523 Partnered (Compugen)	NSCLC	147	<ul style="list-style-type: none"> Open-label, non-randomised dose-escalation and dose-expansion trial: Part A: Dose escalation in checkpoint inhibitor (CPI) experienced NSCLC pts with AZD2936 Intravenous monotherapy Part B: Dose expansion in checkpoint inhibitor (CPI) experienced NSCLC pts with AZD2936 intravenous monotherapy Part C: Dose expansion in CPI Naive NSCLC pts with AZD2936 i.v. monotherapy Part D: Dose expansion. Design to be confirmed by protocol amendment Europe, Australia, Taiwan, South Korea and North America 	<ul style="list-style-type: none"> Part A Dose escalation primary endpoints: safety, RP2D, MTD Part B dose expansion primary endpoints: Safety and efficacy (ORR) Part C dose expansion primary endpoints: Safety and efficacy (ORR) Secondary endpoints: PK, PD (Receptor occupancy), efficacy (DCR, DoR, DRR, PFS) 	<ul style="list-style-type: none"> FPCD: Q4 2021 Data anticipated: >2023



AZD4573 (CDK9 inhibitor)

Blood cancers

Trial	Population	Patients	Design	Endpoints	Status
Phase II NCT05140382	R/R Peripheral T-cell lymphoma and R/R classical Hodgkins Lymphoma	90	<ul style="list-style-type: none"> Open label, non-randomised modular dose confirmation and expansion study in pts with relapsed/ refractory PTCL or cHL Module 1: Exploring AZD4573 monotherapy treatment in 3 cohorts: <ul style="list-style-type: none"> Cohort 1: PTCL, all comers (excluding NKTCL) Cohort 2: PTCL (NKTCL only) Cohort 3: cHL i.v. Route of administration ~30 centres in 10 countries across North America, Europe, EU and ROW 	<ul style="list-style-type: none"> Primary endpoints: Efficacy Secondary endpoint: Safety, PK 	<ul style="list-style-type: none"> FPCD: Q4 2021 LPD: Q3 2023 Data anticipated: >2023
Phase I/II NCT04630756	R/R haematologic malignancies	90	<ul style="list-style-type: none"> Open label, non-randomised trial Module 1 Part A: Dose setting AZD4573 + <i>Calquence</i> (100mg twice daily) combination in DLBCL, all comers; ramp-up across 3 dose levels Module 1 Part B: Dose expansion AZD4573 + <i>Calquence</i> (100mg twice daily) combination in GCB and non-GCB DLBCL Module 2 Part A: Dose Confirmation AZD4573 monotherapy window followed by AZD4573 + acalabrutinib in patients with relapsed or refractory MCL i.v. route of administration 10 countries across North America, EU, ROW 	<ul style="list-style-type: none"> Primary endpoint: Part A: safety Primary endpoint: Part B: ORR Secondary endpoints: Safety, PK, anti-tumour activity 	<ul style="list-style-type: none"> FPCD: Q1 2021 Data anticipated: >2023
Phase I NCT03263637	R/R haematologic malignancies	44	<ul style="list-style-type: none"> Arm 1: dose escalation in haematological malignancies excluding AML/ALL/high-risk MDS/CMML/CLL Arm 2: dose escalation in relapsed or refractory AML, ALL, high-risk MDS, CMML, CLL and Richter's syndrome i.v. route of administration Open in Netherlands, UK, Germany 	<ul style="list-style-type: none"> Primary endpoints: safety, PK Secondary endpoint: efficacy 	<ul style="list-style-type: none"> FPCD: Q4 2017 LPD: Q3 2021 Data anticipated: H2 2022



AZD5305 (PARP1 inhibitor)

Solid tumours

Trial	Population	Patients	Design	Endpoints	Status
Phase I/IIa PETRA NCT04644068	Advanced, metastatic HER2- breast cancer (BRCAm, PALB2m or RAD51C/Dm) Advanced, metastatic TNBC PSR ovarian cancer (BRCAm, PALB2m or RAD51C/Dm) PSR ovarian cancer (HRD+) Prostate cancer (mCRPC, BRCAm) Prostate cancer (mCRPC, HRRm)	715	<ul style="list-style-type: none"> A modular, open-label, multicentre trial dose escalation Module 1: AZD5305 Module 2: AZD5305 + paclitaxel Module 3: AZD5305 + carboplatin +/- paclitaxel Module 4: AZD5305 + trastuzumab deruxtecan Module 5: AZD5305 + datopotamab deruxtecan 	<ul style="list-style-type: none"> Primary endpoints: safety and tolerability, PK Secondary endpoints: efficacy 	<ul style="list-style-type: none"> FPCD: Q4 2020 Data anticipated: >2023
Phase I/IIa PETRANHA NCT05367440	Patients with metastatic prostate cancer	126	<ul style="list-style-type: none"> Multi-arm, open-label, non-randomised, multi-centre study of AZD5305 in combination with physicians' choice new hormonal agents in patients with metastatic prostate cancer. Arm 1: AZD5305 in combination with enzalutamide Arm 2: AZD5305 in combination with abiraterone acetate Arm 3: AZD5305 in combination with darolutamide. 	<ul style="list-style-type: none"> Primary endpoints: Safety and Tolerability Secondary endpoints: PK and Efficacy 	<ul style="list-style-type: none"> FPCD: Q2 2022 Data anticipated: >2023



AZD5991 (MCL1 inhibitor)

Blood cancers

Approved medicines

Late-stage development

Early development

Oncology

Trial	Population	Patients	Design	Endpoints	Status
Phase I/Ib/IIa NCT03218683	Relapsed/refractory haematologic malignancies	144	<ul style="list-style-type: none">• Arm 1: AZD5991 dose escalation and expansion in R/R haematological malignancies• Arm 2: AZD5991 + venetoclax combination dose escalation in R/R AML• i.v. route of administration• US only	<ul style="list-style-type: none">• Primary endpoint: safety• Secondary endpoints: PK, efficacy	<ul style="list-style-type: none">• FPCD: Q3 2017• Data anticipated: >2023• Suspended (The trial has been put on hold to allow further evaluation of safety related information)

CVRM

R&I

Other

V&I

Rare Disease



AZD7789 (PD-1/TIM3 Bispecific mAb)

Cancer

Trial	Population	Patients	Design	Endpoints	Status
Phase I/IIa NCT04931654	NSCLC Other tumours	81	<ul style="list-style-type: none"> Open-label, non-randomised dose-escalation and dose-expansion trial: Part A: Dose escalation in post IO NSCLC pts with AZD7789 intravenous (iv) monotherapy Part B: Dose expansion in post IO and IO naïve NSCLC pts with AZD7789 iv monotherapy. North America, Europe 	<ul style="list-style-type: none"> Primary endpoints: AE, SAE, DLTs, ORR Secondary endpoints: ORR, DCR, DoR, PFS, OS, PK, ADA 	<ul style="list-style-type: none"> FPCD: Q4 2021 Data anticipated: >2023
Phase I/II NCT05216835	Relapsed or Refractory Classical Hodgkin Lymphoma	180	<ul style="list-style-type: none"> Cohort A: Dose Escalation - Patients with anti-PD-1/PD-L1 exposed r/r cHL will receive AZD7789 Cohort B1: Dose Expansion - Patients with anti-PD-1/PD-L1 exposed r/r cHL will receive AZD7789 once the recommended phase 2 dose (RP2D) has been determined Cohort B2: Dose Expansion - Patients with anti-PD-1/PD-L1 naïve r/r cHL will receive AZD7789 once the RP2D has been determined 	<ul style="list-style-type: none"> Primary endpoints: Cohort A: AE and DLTs Cohort B1: AE and ORR Cohort B2: AE and CRR Secondary endpoints: Cohort A: CRR, ORR, DoR, DoCR, PFS, OS, ADA and PK Cohort B1 and B2: DoR, DoCR, PFS, OS, ADA and PK 	<ul style="list-style-type: none"> FPCD: Q2 2022 Data anticipated: >2023



AZD8205 (B7H4 ADC)

Cancer

Approved medicines

Late-stage development

Early development

Oncology

Trial	Population	Patients	Design	Endpoints	Status
Phase I/II NCT05123482	Breast Cancer, Cholangiocarcinoma, Ovarian Cancer, Endometrial Cancer	198	<ul style="list-style-type: none">Open-label, non-randomised dose-escalation and dose-expansion trial	<ul style="list-style-type: none">Primary endpoints: AE, SAE, DLTs, Changes in Lab and PE parametersSecondary endpoints: ORR, DCR, DoR, PFS, OS, PK, ADA	<ul style="list-style-type: none">FPCD: Q1 2022Data anticipated: >2023

CVRM

R&I

Other

V&I

Rare Disease



AZD8701 (FOXP3 antisense oligonucleotide)

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/Ib NCT04504669 Partnered (Ionis)	Advanced solid tumours	153	<ul style="list-style-type: none">Dose escalation and dose expansion trialArm 1: AZD8701 monotherapyArm 2: AZD8701 & Durvaluamb combination therapyi.v. route of administrationUS, CA, FR, ES	<ul style="list-style-type: none">Primary endpoints: safety & tolerabilitySecondary endpoints: PK, PD, preliminary anti-tumour activity	<ul style="list-style-type: none">FPCD: Q3 2020Data anticipated: >2023



AZD8853 (anti-GDF15)

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 NCT05397171	Advanced, unresectable or metastatic NSCLC, Microsatellite Stable Colorectal Cancer, Urothelial Carcinoma	165	<ul style="list-style-type: none">• Open label trial• AZD8853 +/- zirconium-89 crefmirimab berdoxam• Part A: Dose escalation,• Part B: Safety expansion and exploratory CD8+ T cell radiopharmaceutical tracer with PET imaging,• Part C: Efficacy expansion	<ul style="list-style-type: none">• Primary endpoints: safety and tolerability• Secondary endpoints: ORR, DCR, DoR, PFS	<ul style="list-style-type: none">• FPCD: Q3 2022• Data anticipated: >2023



Ceralasertib (AZD6738, ATR inhibitor)

Cancer

Approved medicines

Late-stage development

Early development

Trial	Population	Patients	Design	Endpoints	Status
Phase II PLANETTE NCT04564027	Solid tumours mCRPC	61	<ul style="list-style-type: none"> Cohort A: ceralasertib; ATM-altered AST Cohort B: ceralasertib; ATM-altered mCRPC North America and Europe 	<ul style="list-style-type: none"> Cohort A primary endpoint: ORR Cohort B primary endpoint: Composite RR 	<ul style="list-style-type: none"> FPCD: Q1 2021 Data anticipated: >2023
Phase I 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> North America, Europe and South Korea 	<ul style="list-style-type: none"> Primary endpoints: safety and tolerability. PK and efficacy 	<ul style="list-style-type: none"> FPCD: Q4 2014 Data anticipated: >2023

Oncology

CVRM

R&I

Other

V&I

Rare Disease



IPH5201 (CD39 mAb)

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 NCT04261075 Partnered (TBC)	Advanced solid tumours	204	<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 MTDPart 2: IPH5201 + <i>Imfinzi</i> dose escalation to MTDPart 3: IPH5201 + <i>Imfinzi</i> + oleclumab dose escalation to MTDRoute of administration: i.v.4 countries - US and 3 in EU.	<ul style="list-style-type: none">Primary endpoints: AE, SAE, DLTSecondary endpoints: OR, DC, PK, ADA	<ul style="list-style-type: none">FPCD: Q1 2020Data anticipated: H2 2022



MEDI1191 (IL-12 modRNA)

Cancer

Approved medicines

Late-stage development

Early development

Oncology

Trial	Population	Patients	Design	Endpoints	Status
Phase I NCT03946800 Partnered (Moderna)	Advanced solid tumours	87	<ul style="list-style-type: none">Open-label, dose-escalation and expansion trial of MEDI1191 administered intratumourally as monotherapy and in combination with <i>Imfinzi</i>	<ul style="list-style-type: none">Primary endpoints: safety and tolerabilitySecondary endpoints: PK, immunogenicity and efficacy	<ul style="list-style-type: none">FPCD: Q2 2019Data anticipated: >2023

CVRM

R&I

Other

V&I

Rare Disease



MEDI5752 (PD-1/CTLA-4 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 Ib NCT04522323	Advanced renal cell carcinoma	70	<ul style="list-style-type: none"> Open-label, dose-escalation and dose-expansion trial Arm 1: MEDI5752 and axitinib Arm 2: MEDI5752 and lenvatinib 	<ul style="list-style-type: none"> Dose escalation primary endpoints: safety, MTD, RP2D & tolerability. Assess antitumour activity of the combination (ORR) Secondary endpoints: PK, ADA and antitumour activity (PFS, OR, DoR, DCR, TTR, OS) 	<ul style="list-style-type: none"> FPCD: Q3 2020 Data anticipated: >2023
Phase I NCT03530397	Advanced solid tumours	366	<ul style="list-style-type: none"> Open-label, dose-escalation and dose-expansion trial Dose escalation: MEDI5752 i.v. Dose expansion: MEDI5752 i.v. as monotherapy and in combination with chemotherapy Arm A: MEDI5752 i.v. Arm B: MEDI5752 i.v., pemetrexed and carboplatin Arm C: pembrolizumab, pemetrexed and carboplatin Arm D: MEDI5752 i.v., taxane (paclitaxel or nab-paclitaxel) and carboplatin 	<ul style="list-style-type: none"> Dose escalation primary endpoints: safety, tolerability, MTD, OBD and HPDD. Dose expansion primary endpoint: antitumour activity based on OR Secondary endpoints: PK, ADA, tumoral baseline PD-L1, antitumour activity (OR, DoR, DCR, PFS, OS) 	<ul style="list-style-type: none"> FPCD: Q2 2018 Data anticipated: >2023



MEDI9253 (rNDV-IL-12)

Solid tumours

Approved medicines

Late-stage development

Early development

Oncology

Trial	Population	Patients	Design	Endpoints	Status
Phase I NCT04613492	Advanced solid tumours	86	<ul style="list-style-type: none">Open-label, dose-escalation and expansion trialMEDI9253 + <i>Imfinzi</i>	<ul style="list-style-type: none">Primary endpoint: safety and tolerabilitySecondary endpoints: PK, PD, immunogenicity and efficacy	<ul style="list-style-type: none">FPCD: Q4 2020Data anticipated: >2023

CVRM

R&I

Other

V&I

Rare Disease



Oleclumab (CD73 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 Ib/II Study 5 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.• US, Norway, Spain and Australian trial centres	<ul style="list-style-type: none">• Primary endpoints: safety and anti-tumour activity• Secondary endpoints include: PFS, PK, immunogenicity, safety and anti-tumour activity	<ul style="list-style-type: none">• FPCD: Q2 2018• Data anticipated: H1 2023



**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 hemostatic efficacy as rated by an independent adjudication committee Secondary endpoint: change from baseline in anti-fXa activity 	<ul style="list-style-type: none"> FPCD: Q2 2019 Data anticipated: >2023
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 hemostatic 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 anticipated: H2 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	Patients with 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 anticipated: H2 2023
Phase I NCT04856007	Healthy Chinese volunteers	80	<ul style="list-style-type: none"> Arm 1: <i>Farxiga</i> 5 mg + metformin 500 mg XR Arm 2: <i>Farxiga</i>/metformin XR FDC 5/500 mg Arm 3: <i>Farxiga</i> 10 mg + metformin 1000 mg XR Arm 4: <i>Farxiga</i>/metformin XR FDC 10/1000 mg China only 	<ul style="list-style-type: none"> Primary endpoint: Plasma AUCinf, AUClast and Cmax of <i>Farxiga</i> and metformin respectively. 	<ul style="list-style-type: none"> FPCD: Q2 2021 LPCD: Q2 2021 Data readout: Q4 2021
Phase I NCT05266404	Healthy volunteers	46	<ul style="list-style-type: none"> Arm 1: <i>Farxiga</i> 10 mg + sitagliptin 100 mg Arm 2: <i>Farxiga</i>/sitagliptin FDC 10/100 mg 	<ul style="list-style-type: none"> Primary endpoint: AUCinf, AUClast and Cmax of <i>Farxiga</i> and sitagliptin respectively. 	<ul style="list-style-type: none"> FPCD: Q2 2022 LPCD: Q2 2022 Data anticipated: H2 2022



Lokelma (sodium zirconium cyclosilicate)

Hyperkalaemia

Trial	Population	Patients	Design	Endpoints	Status
Phase IIIb DIALIZE China NCT04217590	Patients with ESRD with hyperkalaemia and on stable haemodialysis	134	<ul style="list-style-type: none"> Arm 1: <i>Lokelma</i> 5g QD for 8 weeks on non-dialysis days. Option to uptitrate to 10 and 15g QD. Arm 2: placebo QD for 8 weeks on non-dialysis days China 	<ul style="list-style-type: none"> Primary endpoint: proportion of patients who maintain a pre-dialysis serum K between 4.0-5.0 mmol/L on 3 out of 4 dialysis treatments following the long interdialytic interval 	<ul style="list-style-type: none"> FPCD: Q4 2020 LPCD: Q3 2021 Data readout: Q1 2022 Primary endpoint met
Phase III HARMONIZE Asia NCT03528681	Hyperkalaemia	250	<ul style="list-style-type: none"> Open-label <i>Lokelma</i> 10g TID for 48 hours followed by: Arm 1: <i>Lokelma</i> 5g QD for 28 days Arm 2: <i>Lokelma</i> 10g QD for 28 days Arm 3: placebo QD for 28 days China 	<ul style="list-style-type: none"> Primary endpoint: maintenance of normokalaemia 	<ul style="list-style-type: none"> FPCD: Q2 2021 Data anticipated: H2 2022
Phase III DIALIZE-Outcomes NCT04847232	Patients with recurrent hyperkalaemia on chronic haemodialysis	2300	<ul style="list-style-type: none"> Arm 1: <i>Lokelma</i> 5g-15g QD for 4 weeks on non-dialysis days, thereafter adjusted monthly Arm 2: placebo QD Global trial – 22 countries 	<ul style="list-style-type: none"> Primary endpoint: Time to first occurrence of SCD, stroke, or hospitalisation/intervention/ED visit due to arrhythmias 	<ul style="list-style-type: none"> FPCD: Q3 2021 Data anticipated: >2023
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 72h, 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 randomized to a 24 month treatment: Arm 1: <i>Lokelma</i> (5g QOD to 15g QD) and lisinopril or valsartan Arm 2: Placebo and lisinopril or valsartan Global trial – 13 countries 	<ul style="list-style-type: none"> Primary endpoint: Total slope (eGFR measurements starting at randomisation) and chronic slope (eGFR measurements starting at 12 weeks after randomisation) 	<ul style="list-style-type: none"> Data anticipated: >2023



Roxadustat (HIF-PH inhibitor)

Anaemia

Trial	Population	Patients	Design	Endpoints	Status
Phase III Efficacy and Safety of FG-4592 for Treatment of Anemia in Patients With Lower Risk MDS With Low Red Blood Cell Transfusion Burden NCT03263091 Partnered (FibroGen)	Anaemia in lower risk MDS patients	204	<ul style="list-style-type: none"> Open label roxadustat lead-in Arm 1: roxadustat Arm 2: placebo US/global trial 	<ul style="list-style-type: none"> Primary endpoint: proportion of patients achieving transfusion independence 	<ul style="list-style-type: none"> FPCD: Q3 2017 Data anticipated: H1 2023 Sponsored by FibroGen
Phase II/III Efficacy and Safety of FG-4592 for Treatment of Anemia in Subjects With Lower Risk MDS NCT03303066 Partnered (FibroGen)	Anaemia in lower risk MDS patients	175	<ul style="list-style-type: none"> Open label roxadustat lead-in Arm 1: roxadustat Arm 2: placebo China 	<ul style="list-style-type: none"> Primary endpoint: haemoglobin response 	<ul style="list-style-type: none"> FPCD: Q2 2018 Data anticipated: H1 2023 Sponsored by FibroGen
Phase II Evaluation of Efficacy and Safety of Roxadustat for the Treatment of Chemotherapy Induced Anemia NCT04076943 Partnered (FibroGen)	Anaemia in patients receiving chemotherapy treatment for non-myeloid malignancies	92	<ul style="list-style-type: none"> Open label trial roxadustat 3x week 16 weeks US 	<ul style="list-style-type: none"> Primary endpoint: maximum change in haemoglobin within 16 weeks from baseline without RBC transfusion 	<ul style="list-style-type: none"> FPCD: Q3 2019 LPCD: Q3 2020 Data readout: Q2 2021 Primary endpoint met Sponsored by FibroGen



AZD4831 (MPO inhibitor)

Cardiovascular disease

Trial	Population	Patients	Design	Endpoints	Status
Phase IIb/III ENDEAVOR NCT04986202	HFpEF	1485	<ul style="list-style-type: none"> Randomised, double blind Arm 1: 2.5mg AZD4831 Arm 2: 5mg AZD4831 Arm 3: placebo Global trial 	<ul style="list-style-type: none"> Endpoints: Efficacy and Safety 	<ul style="list-style-type: none"> FPCD: Q3 2021 Data anticipated: >2023
Phase IIa SATELLITE NCT03756285	HFpEF	96	<ul style="list-style-type: none"> Arm 1: AZD4831 Arm 2: placebo Global trial – five countries 	<ul style="list-style-type: none"> Primary endpoint: The change from baseline in MPO activity in % 	<ul style="list-style-type: none"> FPCD: Q4 2018 Data readout: Q2 2021
Phase I NCT02712372	Healthy patients	96	<ul style="list-style-type: none"> SAD trial Arm 1: AZD4831 Arm 2: placebo Germany 	<ul style="list-style-type: none"> Primary endpoints: safety and tolerability Secondary endpoint: PK parameters 	<ul style="list-style-type: none"> FPCD: Q3 2016 LPCD: Q4 2016 Data readout: Q2 2017
Phase I NCT03136991	Healthy patients	40	<ul style="list-style-type: none"> MAD trial Arm 1: AZD4831 Arm 2: placebo USA 	<ul style="list-style-type: none"> Primary endpoints: safety and tolerability Secondary endpoint: PK parameters 	<ul style="list-style-type: none"> FPCD: Q2 2017 LPCD: Q4 2017 Data readout: Q1 2018
Phase I NCT04232345	Healthy patients	32	<ul style="list-style-type: none"> MAD trial in Japanese and Chinese patients 	<ul style="list-style-type: none"> Primary endpoints: safety and tolerability 	<ul style="list-style-type: none"> FPCD: Q1 2020 Data readout: Q3 2021
Phase I NCT04407091	Healthy patients	6	<ul style="list-style-type: none"> Open label hADME trial a single oral dose of [¹⁴C] AZD4831 UK 	<ul style="list-style-type: none"> Primary endpoints: mass balance, with routes and rates of elimination of [¹⁴C]AZD4831; Metabolite profiling and structural identification; PK and total radioactivity 	<ul style="list-style-type: none"> FPCD: Q2 2020 LPCD: Q3 2020 Data readout: Q3 2021
Phase I NCT05052710	Healthy patients	14	<ul style="list-style-type: none"> Open label AZD4831 AZD4831 and Midazolam UK 	<ul style="list-style-type: none"> Primary endpoints: PK parameters Secondary endpoints: safety and tolerability 	<ul style="list-style-type: none"> FPCD: Q4 2021 LPCD: Q4 2021 Data readout: Q1 2022
Phase I NCT04949438	Renal Impairment	20	<ul style="list-style-type: none"> Open label AZD4831 	<ul style="list-style-type: none"> Primary endpoints: PK parameters Secondary endpoints: safety and tolerability 	<ul style="list-style-type: none"> FPCD: Q1 2022 LPCD: Q1 2022 Data readout: Q2 2022



eplontersen (ligand-conjugated antisense)

ATTR

Approved medicines

Late-stage development

Early development

Trial	Population	Patients	Design	Endpoints	Status
Phase III CARDIO-TTRansform NCT04136171 Partnered (Ionis Pharmaceuticals, Inc.)	Patients with hereditary or wild-type Transthyretin-Mediated Amyloid Cardiomyopathy (ATTR CM)	750	<ul style="list-style-type: none"> • Arm 1: eplontersen s.c. • Arm 2: placebo 	<ul style="list-style-type: none"> • Primary endpoint: composite Outcome of Cardiovascular (CV) Mortality and recurrent CV clinical events at Week 120 • Secondary endpoints include: 6MWT, KCCQ, CV Events, CV Mortality 	<ul style="list-style-type: none"> • FPCD: Q1 2020 • Data anticipated: >2023
Phase III NEURO-TTRansform NCT04136184 Partnered (Ionis Pharmaceuticals, Inc.)	Patients with 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 endpoint: change from baseline in mNIS+7 at Week 66 	<ul style="list-style-type: none"> • FPCD: Q1 2020 • Data readout: Q2 2022 • Met co-primary endpoints

Oncology

CVRM

R&I

Other

V&I

Rare Disease



Tozorakimab (IL-33 ligand mAb)

Diabetic 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 II NCT04170543	Adult patients with diabetic kidney disease	565	<ul style="list-style-type: none">• Arm A: tozorakimab dose 1 + <i>Farxiga</i>• Arm B: tozorakimab dose 2 + <i>Farxiga</i>• Arm C: tozorakimab dose 3 + <i>Farxiga</i>• Arm D: tozorakimab dose 4 + <i>Farxiga</i>• Arm E: placebo + <i>Farxiga</i>• USA, Canada, Japan and additional countries.	<ul style="list-style-type: none">• Primary endpoint: Urine albumin creatinine ratio (UACR)• Secondary endpoints: Safety and other efficacy measures	<ul style="list-style-type: none">• FPCD: Q4 2019• Data anticipated: H1 2023



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

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 multicentre 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 Multi-country 	<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 24 Primary endpoint of pooled trials D5982C00007 and D5982C00008: Rate of severe asthma exacerbations Secondary endpoint: Change from baseline in morning pre-dose trough FEV1 at Week 24 	<ul style="list-style-type: none"> FPCD: Q1 2021 Data anticipated: >2023
Phase III LOGOS NCT04609904	Severe asthma	2200	<ul style="list-style-type: none"> Randomised, double-blind, double dummy, parallel group and multicentre 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 Multi-country 	<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 24 Primary endpoint of pooled trials D5982C00007 and D5982C00008: Rate of severe asthma exacerbations Secondary endpoint: Change from baseline in morning pre-dose trough FEV1 at Week 24 	<ul style="list-style-type: none"> FPCD: Q1 2021 Data anticipated: >2023
Phase III VATHOS NCT05202262	Moderate asthma	630	<ul style="list-style-type: none"> Randomized, double-blind, parallel group and multicentre 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 Multi-country 	<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 24 	<ul style="list-style-type: none"> FPCD: Q1 2022 Data anticipated: >2023



Daliresp/Daxas (PDE4 inhibitor, oral)

COPD

Approved medicines
Late-stage development
Early development

Trial	Population	Patients	Design	Endpoints	Status
Phase IV PASS (post launch) NCT03381573	COPD	124080	<ul style="list-style-type: none">A retrospective cohort trial comparing COPD patients aged 40 years and older with new exposure to roflumilast with up to 5 unexposed (i.e., not roflumilast-exposed) COPD controls matched by propensity score (PS), age, sex, and year of cohort entry.The trial is using electronic healthcare databases in the US (Military Health System database), Germany (German Pharmacoepidemiological Research Database), Sweden (national databases including healthcare, death, and demographics data) and Norway.	<ul style="list-style-type: none">Primary endpoint: all-cause mortality (up to five years)	<ul style="list-style-type: none">FPCD: Q1 2017LPCD: Q4 2022Data anticipated: H2 2022

Oncology
CVRM
R&I
Other
V&I
Rare Disease



Fasenra (IL-5R mAb)

Dermatology

Approved medicines

Late-stage development

Early development

Trial	Population	Patients	Design	Endpoints	Status
Phase III FJORD NCT04612790	Patients with symptomatic (newly diagnosed or relapsing) bullous pemphigoid	120	<ul style="list-style-type: none"> • Double blind treatment period and open label period • Arm 1: <i>Fasenra</i> • Arm 2: placebo • 36-week • Global trial 	<ul style="list-style-type: none"> • Primary endpoint: Proportion of patients with complete sustained (≥ 2 months) remission off OCS at 36 weeks 	<ul style="list-style-type: none"> • FPCD: Q2 2021 • Data anticipated: >2023
Phase II ARROYO NCT04612725	Patients with moderate/severe chronic spontaneous urticaria, and resistant to H1 treatment	155	<ul style="list-style-type: none"> • Double blind treatment period and open label period • Arm 1: <i>Fasenra</i> regimen 1 • Arm 2: <i>Fasenra</i> regimen 2 • Arm 3: placebo • 24-week • Global trial 	<ul style="list-style-type: none"> • Primary endpoint: Change from baseline in ISS7 at week 12 	<ul style="list-style-type: none"> • FPCD: Q4 2020 • LPCD: Q2 2022 • Data anticipated: H2 2022
Phase II HILLIER NCT04605094	Patients with moderate to severe atopic dermatitis despite treatment with topical medications	194	<ul style="list-style-type: none"> • Double blind treatment period and open label periods • Arm 1: <i>Fasenra</i> • Arm 2: placebo • 16-week DB period • Global trial 	<ul style="list-style-type: none"> • Primary endpoint: Proportion of patients with an IGA 0/1 and a decrease in IGA of ≥ 2 points at week 16 	<ul style="list-style-type: none"> • FPCD: Q4 2020 • LPCD: Q4 2021 • Data readout: Q2 2022 • Did not meet primary endpoint

Oncology

CVRM

R&I

Other

V&I

Rare Disease



Fasenra (IL-5R mAb)

Gastrointestinal diseases

Trial	Population	Patients	Design	Endpoints	Status
Phase III MESSINA NCT04543409	Documented diagnosis of EoE Age 12 to 65 years	211	<ul style="list-style-type: none"> • Double blind treatment period and open label period(s) • Arm 1: <i>Fasenra</i> 30mg Q4W s.c. • Arm 2: placebo Q4W s.c. • 24-week • Global trial – 12 countries 	<ul style="list-style-type: none"> • Primary endpoints: histologic response at week 24, change from baseline in DSQ score at week 24 	<ul style="list-style-type: none"> • FPCD: Q4 2020 • LPCD: Q2 2022 • Data anticipated: H2 2022
Phase III HUDSON NCT05251909	Patients with eosinophilic gastritis and/or gastroenteritis. Age >=12yrs	220	<ul style="list-style-type: none"> • Double blind treatment period and open label extension • Arm 1: <i>Fasenra</i> s.c. • Arm 2: placebo s.c. • 24-week • Global trial 	<ul style="list-style-type: none"> • Dual primary endpoints at week 24: • Proportion of patients achieving a histological response in the stomach and/or in the duodenum • Absolute change in symptoms of EG/EGE 	<ul style="list-style-type: none"> • FPCD: Q1 2022 • Data anticipated: >2023



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 standard of care therapy Age 18-75 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 18-75 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 endpoint: 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: >2023
Phase III MANDARA NCT04157348	Patients with relapsing or refractory EGPA on corticosteroid therapy with or without stable immunosuppressive therapy Age 18 years and older	140	<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 \leq 4mg/day) at both weeks 36 and 48. 	<ul style="list-style-type: none"> FPCD: Q4 2019 Data anticipated: H2 2023
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- 9-12 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: H2 2023



Fasenra (IL-5R mAb)

Severe, uncontrolled asthma

Trial	Population	Patients	Design	Endpoints	Status
Phase IIIb PONENTE NCT03557307	Severe eosinophilic asthmatics receiving HD ICS + LABA and chronic OCS with or without additional asthma controller(s). Age 18 Years and older	599	<ul style="list-style-type: none"> Arm 1: <i>Fasenra</i> 30mg Q8W s.c. 38-week trial Global trial – 16 countries 	<ul style="list-style-type: none"> Primary endpoint: reduction of oral corticosteroid dose 	<ul style="list-style-type: none"> FPCD: Q3 2018 LPCD: Q3 2019 Data readout: Q4 2020 Primary endpoint met
Phase III D3250C00036 China ICS/LABA Trial (MIRACLE) NCT03186209	Severe, uncontrolled asthma, despite background controller medication, MD & HD ICS + LABA ± chronic OCS Age 12-75 years	695	<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 – 4 countries 	<ul style="list-style-type: none"> Primary endpoint: annual asthma exacerbation rate Secondary endpoints: assess pulmonary function, asthma symptoms, other asthma control metrics 	<ul style="list-style-type: none"> FPCD: Q4 2017 LPCD: Q4 2021 Data anticipated: H1 2023



Fasenra (IL-5R mAb)

Severe, uncontrolled asthma, COPD and other eosinophilic diseases

Trial	Population	Patients	Design	Endpoints	Status
Phase III RESOLUTE NCT04053634	Patients with moderate to very severe COPD with a history of frequent exacerbations on a background triple therapy (ICS/LABA/LAMA) Age 40-85 years	642	<ul style="list-style-type: none"> • Double-blind, placebo controlled • 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: annualized rate of moderate or severe exacerbations over 56 weeks 	<ul style="list-style-type: none"> • FPCD: Q4 2019 • Data anticipated: >2023
Phase III MAHALE NCT05006573	Patients With non-cystic fibrosis bronchiectasis (NCFB) with eosinophilic inflammation Age 18 years and older	420	<ul style="list-style-type: none"> • Double blind treatment period and open label extension trial • Arm 1: <i>Fasenra</i> 30mg Q4W s.c. • Arm 2: placebo Q4W s.c. • 52-week • Global trial – 17 countries 	<ul style="list-style-type: none"> • Primary endpoint: annualised bronchiectasis exacerbation rate at week 52 	<ul style="list-style-type: none"> • FPCD: Q3 2021 • Data anticipated: >2023



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 LTE NCT02794285 Partnered (BMS)	Moderate to severe SLE patients	630	<ul style="list-style-type: none"> Arm 1: 300mg i.v. <i>Saphnelo</i> Q4W for 152 weeks Arm 2: placebo i.v. Q4W for 152 weeks 	<ul style="list-style-type: none"> Primary endpoint: extension to evaluate long-term safety and tolerability 	<ul style="list-style-type: none"> FPCD: Q2 2016 LPCD: Q4 2018 Data anticipated: H2 2022
Phase III TULIP-SC NCT04877691 Partnered (BMS)	Moderate to severe SLE patients	360	<ul style="list-style-type: none"> Arm 1: <i>Saphnelo</i> s.c. Arm 2: placebo s.c. Global 	<ul style="list-style-type: none"> Primary endpoint: BICLA at week 52 	<ul style="list-style-type: none"> FPCD: Q3 2021 Data anticipated: >2023
Phase III AZALEA-SLE NCT04931563 Partnered (BMS)	Moderate to severe SLE patients	328	<ul style="list-style-type: none"> Arm 1: 300 mg <i>Saphnelo</i> i.v. Q4W Arm 2: placebo i.v. Q4W Asia 	<ul style="list-style-type: none"> Primary endpoint: BICLA at week 52 	<ul style="list-style-type: none"> FPCD: Q4 2021 Data anticipated: >2023
Phase III IRIS NCT05138133 Partnered (BMS)	Active Proliferative LN patients	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"> Complete Renal Response (CRR) at week 52 	<ul style="list-style-type: none"> FPCD: Q2 2022 Data anticipated: >2023

Oncology

CVRM

R&I

Other

V&I

Rare Disease



Tezspire (TSLP mAb)

Severe, uncontrolled asthma

Trial	Population	Patients	Design	Endpoints	Status
Phase III NAVIGATOR NCT03347279 Partnered (AMGEN)	Severe asthma Age 12-80 years	1061	<ul style="list-style-type: none"> Arm 1: Tezspire 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), 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 SOURCE NCT03406078 Partnered (AMGEN)	Severe asthma Age 18-80 years	150	<ul style="list-style-type: none"> Arm 1: Tezspire s.c. Arm 2: placebo s.c. 48 week trial Global trial – seven countries 	<ul style="list-style-type: none"> Primary endpoint: Reduction from baseline in daily OCS dose while not losing asthma control Secondary endpoint: Annual asthma exacerbation rate 	<ul style="list-style-type: none"> FPCD: Q2 2018 LPCD: Q4 2019 Data readout: Q4 2020 Primary endpoint not met
Phase III DESTINATION NCT03706079 Partnered (AMGEN)	Severe asthma Age 12-80 years	951	<ul style="list-style-type: none"> Extension trial to NAVIGATOR and SOURCE Arm 1: Tezspire s.c. Arm 2: placebo s.c. 52 week trial (subjects from NAVIGATOR); 56 week trial (subjects from SOURCE) Global trial – 18 countries 	<ul style="list-style-type: none"> Primary endpoint: Exposure adjusted rates of AEs/SAEs Secondary endpoints: Annual asthma exacerbation rate 	<ul style="list-style-type: none"> FPCD: Q1 2019 LPCD: Q4 2020 Data anticipated: H2 2022
Phase III PATH-HOME NCT03968978 Partnered (AMGEN)	Severe asthma Age 12-80 years	216	<ul style="list-style-type: none"> Arm 1: Tezspire s.c. via AI Arm 2: Tezspire s.c. via APFS 24 week trial Global trial – 4 countries 	<ul style="list-style-type: none"> Primary endpoint: Proportion of health care professionals and patients /caregivers who successfully administrated Tezspire in clinic and at home with an APFS or an AI, respectively 	<ul style="list-style-type: none"> FPCD: Q2 2019 LPCD: Q3 2019 Data readout: Q4 2020 Primary endpoint met



Tezspire (TSLP mAb)

Severe, uncontrolled asthma, COPD & CRSwNP

Trial	Population	Patients	Design	Endpoints	Status
Phase III WAYPOINT NCT04851964 Partnered (AMGEN)	Severe chronic rhinosinusitis with nasal polyps (CRSwNP) Age 18+	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 Data anticipated: >2023
Phase III DIRECTION NCT03927157 Partnered (AMGEN)	Severe asthma Age 18-80 years	396	<ul style="list-style-type: none"> Arm 1: <i>Tezspire</i> s.c. Arm 2: placebo s.c. 52 week trial Regional Asia trial – three 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), asthma control (ACQ-6) 	<ul style="list-style-type: none"> FPCD: Q3 2019 Data anticipated: >2023
Phase III NOZOMI NCT04048343 Partnered (AMGEN)	Severe asthma 12-80 years	65	<ul style="list-style-type: none"> Arm 1: <i>Tezspire</i> s.c. 52 week trial Local trial - Japan 	<ul style="list-style-type: none"> Primary endpoint: number of patients with adverse events 	<ul style="list-style-type: none"> FPCD: Q2 2019 LPCD: Q1 2020 Data readout: Q2 2021 Primary endpoint met
Phase IIa COURSE NCT04039113 Partnered (AMGEN)	Moderate to very severe COPD Age 40-80	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 Data anticipated: >2023
Phase II CASCADE NCT03688074 Partnered (AMGEN)	Severe asthma Age 18-75 years	116	<ul style="list-style-type: none"> Arm 1: <i>Tezspire</i> s.c. Arm 2: placebo s.c. 28 week trial Global trial – five countries 	<ul style="list-style-type: none"> Primary endpoint: number of airway submucosal inflammatory cells/mm² of bronchoscopic biopsies 	<ul style="list-style-type: none"> FPCD: Q4 2018 LPCD: Q4 2019 Data readout: Q2 2021 Primary endpoint met



Brazikumab (IL-23 inhibitor)

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

Trial	Population	Patients	Design	Endpoints	Status
Phase III NCT03961815	Crohn's Disease	161	<ul style="list-style-type: none"> An Open-label, Long-term Extension Safety Study of Brazikumab in Participants With Moderately to Severely Active Crohn's Disease 	<ul style="list-style-type: none"> Primary endpoint: safety of long-term treatment with Brazikumab (AEs, clinical laboratory values, vital signs, and ECGs) 	<ul style="list-style-type: none"> FPCD: Q1 2020 Data anticipated: >2023
Phase IIb/III INTREPID NCT03759288	Crohn's disease	928	<ul style="list-style-type: none"> A 52-Week, Multicenter, Randomized, Double-blind, Placebo and Active-Controlled, Operationally Seamless Phase 2b/3, Parallel group Study to Assess the Efficacy and Safety of Brazikumab in Participants With Moderately to Severely Active Crohn's Disease Stage 1 Arm 1: brazikumab high i.v. dose on day 1, 29 and 57 + s.c. brazikumab on day 85 and every 4 weeks through week 48 Arm 2: brazikumab low i.v. dose on day 1, 29 and 57 s.c. brazikumab on day 85 and every 4 weeks through week 48 Arm 3: placebo Stage 2 Arm 1: brazikumab high i.v. dose on day 1, 29 and 57 + s.c. brazikumab on day 85 and every 4 weeks through week 48 Arm 2: brazikumab low i.v. dose on day 1, 29 and 57 s.c. brazikumab on day 85 and every 4 weeks through week 48 Arm 3: adalimumab s.c. on day 1, 15, 29 and every 2 weeks through week 50 	<ul style="list-style-type: none"> Stage 1 primary endpoint: CDAI remission at week 12 Stage 1 secondary endpoints: Endoscopic response at week 12, Clinical remission at week 12, CDAI response at week 12, Response and remission at week 52 Stage 2 primary endpoints: Endoscopic response at week 52, Clinical remission at week 52 	<ul style="list-style-type: none"> FPCD: Q4 2018 Data anticipated: >2023
Phase II EXPEDITION NCT03616821	Ulcerative colitis	256	<ul style="list-style-type: none"> A 54-Week, Multicenter, Randomized, Double-blind, Placebo controlled, Parallel-group Phase 2 Study to Assess the Efficacy and Safety of Brazikumab in Participants with Moderately to Severely Active Ulcerative Colitis Arm 1: brazikumab dose 1 i.v. on day 1, 15 and 43 + s.c. brazikumab from day 71 and every 4 weeks Arm 2: brazikumab dose 2 i.v. on day 1, 15 and 43 + s.c. brazikumab from day 71 and every 4 weeks Arm 3: placebo 	<ul style="list-style-type: none"> Primary endpoint: clinical remission at week 10 Secondary endpoint: sustained clinical remission at week 10 and 54 	<ul style="list-style-type: none"> FPCD: Q3 2018 Data anticipated: >2023



Brazikumab (IL-23 inhibitor)

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

Trial	Population	Patients	Design	Endpoints	Status
Phase II NCT04277546	Ulcerative colitis	165	<ul style="list-style-type: none"> A Phase 2 Open-label, Long-term Extension Safety Study of Brazikumab in Participants with Moderately to Severely Active Ulcerative Colitis 	<ul style="list-style-type: none"> Primary endpoint: safety of long-term treatment with Brazikumab (AEs, clinical laboratory values, vital signs, and ECGs) 	<ul style="list-style-type: none"> FPCD: Q1 2020 Data anticipated: >2023
Phase I NCT05033431	Healthy volunteers	48	<ul style="list-style-type: none"> Open-label Study to Evaluate the Pharmacokinetics, Safety and Tolerability of a Single Dose of Brazikumab Administered by IV Infusion and SC Injection in Healthy Chinese and White Participants 	<ul style="list-style-type: none"> Primary pharmacokinetic endpoints: C_{max} AUC_{inf} AUC_{last} AUC_{0-28d} 	<ul style="list-style-type: none"> FPCD: Q4 2021 Data anticipated: H2 2022



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, multicentre, parallel group Treatments (minimum 24-week treatment period) BDA (budesonide albuterol) MDI 80/180 µg prn BDA MDI 160/180 µg prn AS (albuterol sulphate) MDI 180 µg prn Multi-country 	<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, multicentre and parallel-group Treatments (12 week treatment period) BDA MDI 80/180 µg QID BDA MDI 160/180 µg QID BD MDI 160 µg QID AS MDI 180 µg QID placebo MDI QID Multi-country 	<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, multicentre crossover Treatments (single dose) BDA MDI 160/180 µg placebo MDI QID US 	<ul style="list-style-type: none"> Primary endpoint: The maximum percentage fall from post-dose, pre-exercise baseline in forced expiratory volume in 1 second (FEV1) observed up to 60 minutes post-exercise challenge 	<ul style="list-style-type: none"> FPCD: Q1 2020 LPCD: Q3 2020 Data readout: Q4 2020 Primary endpoint met



Tozorakimab (IL-33 ligand mAb)

Atopic dermatitis, asthma

Trial	Population	Patients	Design	Endpoints	Status
Phase II NCT04212169	Adult subjects with atopic dermatitis	152	<ul style="list-style-type: none"> Randomised, double-blind, placebo-controlled trial Arm 1: tozorakimab s.c. Arm 2: tozorakimab s.c. Arm 3: tozorakimab s.c. Arm 4: placebo s.c. Global study: 6 countries 	<ul style="list-style-type: none"> Primary endpoint: change from baseline at week 16 in Eczema Area and Severity Index (EASI) score Secondary endpoints: safety and other efficacy measures 	<ul style="list-style-type: none"> FPCD: Q4 2019 Data anticipated: H2 2022
Phase II NCT04570657	Adult participants with uncontrolled moderate to severe asthma	228	<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. 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 Data anticipated: H1 2023
Phase I NCT05070312	Healthy subjects	36	<ul style="list-style-type: none"> Randomized, 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 	<ul style="list-style-type: none"> Primary endpoint: to characterize the pharmacokinetics of tozorakimab Secondary endpoints: to evaluate the immunogenicity of tozorakimab 	<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	1272	<ul style="list-style-type: none"> Randomised, double-blind, placebo-controlled, parallel group Treatment: 52 week Arm 1: Tozorakimab dose 1 s.c. + standard of care Arm 2: Tozorakimab dose 2 s.c. + standard of care Arm 3: Placebo s.c. + standard of care Global trial: 20 countries 	<ul style="list-style-type: none"> Primary endpoint: annualized rate of moderate to severe COPD exacerbations (former smokers) Secondary endpoints: annualized rate of moderate to severe COPD exacerbations (former or current smokers), time to moderate to severe COPD exacerbation, change in pre-BD FEV1, E-RS:COPD and SGRQ 	<ul style="list-style-type: none"> FPCD: Q1 2022 Data anticipated: >2023
Phase III TITANIA NCT05158387	Adults with symptomatic COPD with a history of exacerbations	1272	<ul style="list-style-type: none"> Randomised, double-blind, placebo-controlled, parallel group Treatment: 52 week Arm 1: Tozorakimab dose 1 s.c. + standard of care Arm 2: Tozorakimab dose 2 s.c. + standard of care Arm 3: Placebo s.c. + standard of care Global trial: 19 countries 	<ul style="list-style-type: none"> Primary endpoint: annualized rate of moderate to severe COPD exacerbations (former smokers) Secondary endpoints: annualized rate of moderate to severe COPD exacerbations (former or current smokers), time to moderate to severe COPD exacerbation, change in pre-BD FEV1, E-RS:COPD and SGRQ 	<ul style="list-style-type: none"> FPCD: Q1 2022 Data anticipated: >2023
Phase II NCT04631016	Adult subjects with COPD and chronic bronchitis	144	<ul style="list-style-type: none"> Randomised, double-blind, placebo-controlled, parallel group, proof of concept trial Arm 1: Tozorakimab s.c. Arm 2: placebo s.c. Global study: 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 anticipated: H2 2023



Evusheld (AZD7442, tixagevimab + cilgavimab)

Prevention and treatment of COVID-19

Trial	Population	Patients	Design	Endpoints	Status
Phase III PROVENT NCT04625725	Adults having increased risk for inadequate response to active immunisation or having increased risk for SARS-CoV-2 infection	5197	<ul style="list-style-type: none"> • Double-blinded, randomised, placebo controlled, multi centre study to determine safety and efficacy in pre-exposure prophylaxis • Arm 1: <i>Evusheld</i> • Arm 2: placebo • <i>Evusheld</i>/placebo (2:1) • USA, UK, Belgium, France, Spain 	<ul style="list-style-type: none"> • Primary endpoint: positive symptomatic illness post –dose • Secondary endpoints: Incidence of: nucleocapsid antibodies, emergency visits, PCR positive, ADA to <i>Evusheld</i> in serum and <i>Evusheld</i> serum conc. 	<ul style="list-style-type: none"> • FPCD: Q4 2020 • LPCD: Q1 2021 • Data readout: Q3 2021 • Primary endpoint met
Phase III STORM CHASER NCT04625972	Adults with potential exposure to an identified individual with confirmed SARS-COV2 infection and at risk of developing COVID-19	1121	<ul style="list-style-type: none"> • Double-blinded, randomised, placebo controlled, multi centre study to determine safety and efficacy in post-exposure prophylaxis • Arm 1: <i>Evusheld</i> • Arm 2: placebo • <i>Evusheld</i>/placebo (2:1) • USA and UK 	<ul style="list-style-type: none"> • Primary endpoint: positive symptomatic illness post –dose • Secondary endpoints: Incidence of: nucleocapsid antibodies, COVID-19 related death, all cause mortality, ADA to <i>Evusheld</i> in serum and AZD7442 serum conc. 	<ul style="list-style-type: none"> • FPCD: Q4 2020 • LPCD: Q1 2021 • Data readout: Q2 2021 • Primary endpoint not met
PHASE III TACKLE NCT04723394	Adults with confirmed mild to moderate SARS-COV2 infection. Symptomatic patients with documented positive SARS-Cov-2 molecular test	910	<ul style="list-style-type: none"> • Double-blinded, randomised, placebo controlled, multi centre study to determine safety and efficacy for treatment of Covid-19 in non-hospitalised patients • Arm 1: <i>Evusheld</i> • Arm 2: placebo • <i>Evusheld</i>/placebo (1:1) • UK, Germany, Spain, Italy, Hungary, Russia, US, Mexico, Japan, Poland, Czech Republic, Argentina, Brazil, and Ukraine 	<ul style="list-style-type: none"> • Primary endpoint: efficacy in the prevention of the composite endpoint of either severe COVID-19 or death from any cause through study day 29 • Secondary endpoints: A composite of either death from any cause or hospitalisation for COVID-19 complications or sequelae (Day 1 to Day 169). Determine symptom severity and prevention of respiratory failure 	<ul style="list-style-type: none"> • FPCD: Q1 2021 • LPCD: Q3 2021 • Data readout: Q4 2021 • Primary endpoint met
Phase I NCT04507256	Healthy adults Aged 18-55 years	60	<ul style="list-style-type: none"> • Double-blinded, randomised, placebo controlled, single ascending dose trial • Arm 1: <i>Evusheld</i> • Arm 2: placebo • <i>Evusheld</i>/placebo (10:2) • UK 	<ul style="list-style-type: none"> • Primary endpoint: safety, tolerability and PK • Secondary endpoint: immunogenicity 	<ul style="list-style-type: none"> • FPCD: Q3 2020 • LPCD: Q3 2020 • Data readout: Q1 2022



Evusheld (AZD7442, tixagevimab + cilgavimab)

Prevention and treatment of COVID-19

Trial	Population	Patients	Design	Endpoints	Status
Phase I PK Co-formulation NCT05166421	Healthy adults Aged ≥ 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) USA 	<ul style="list-style-type: none"> Key Endpoints: safety and PK 	<ul style="list-style-type: none"> FPCD: Q1 2022 Data anticipated: H2 2023
Phase I TRUST NCT05281601 Partnered	Pediatric participants aged ≥ 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 yet to open) Cohort 3: Severe COVID-19 (cohort yet to open) <i>Evusheld</i> USA 	<ul style="list-style-type: none"> Key endpoints: Safety, tolerability and PK 	<ul style="list-style-type: none"> FPCD: Q1 2022 Data anticipated: H2 2022



Vaxzevria (SARS-CoV-2)

Prevention of COVID-19

Trial	Population	Patients	Design	Endpoints	Status
Phase IV VICTORIA NCT05057897	Seronegative immunocompromised and immunocompetent individuals who are unvaccinated	360	<ul style="list-style-type: none"> Open-label, non-randomized, multi-cohort, multicenter trial. Vaxzevria i.m. Arm 1: Solid organ transplant Arm 2: Haematopoietic stem cell transplant Arm 3: Solid organ cancer patients receiving cytotoxic therapy Arm 4: Chronic inflammatory disorders Arm 5: Primary immunodeficiency Arm 6: Immunocompetent 	<ul style="list-style-type: none"> Immunogenicity and safety 	<ul style="list-style-type: none"> FPCD: Q1 2022 LPCD: Q3 2022 Data anticipated: H2 2023
Phase III D8110C00001 (US, global) NCT04516746	Healthy adults Aged 18-65 years	32429	<ul style="list-style-type: none"> Adaptive, double-blinded, randomised placebo-controlled trial Vaxzevria placebo US, Peru, Chile 	<ul style="list-style-type: none"> Primary endpoints: efficacy, safety, tolerability, and reactogenicity Secondary endpoints: immunogenicity 	<ul style="list-style-type: none"> FPCD: Q3 2020 LPCD: Q1 2021 Primary data readout Q1 2021
Phase III COV003 (Brazil) NCT04536051 Partnered (Oxford University)	Health professionals and adults with high potential for exposure to SARS-CoV-2 Age 18-55 years	10416	<ul style="list-style-type: none"> Single-blinded, randomised, controlled multicentre trial Vaxzevria Control vaccine: MenACWY Brazil 	<ul style="list-style-type: none"> Primary endpoint: efficacy Secondary endpoints: safety, tolerability, reactogenicity, and immunogenicity 	<ul style="list-style-type: none"> FPCD: Q2 2020 LPCD: Q4 2020
Phase II/III COV002 (UK) NCT04400838 Partnered (Oxford University)	Main efficacy trial: healthy adults aged ≥18 years Healthy adults 56 - <70 years Healthy adults ≥70 years Healthy children 5 – 12 years	10812	<ul style="list-style-type: none"> Single-blinded, randomised, controlled, multicentre trial with sequential age escalation/de-escalation immunogenicity sub-studies that include prime boost Vaxzevria Control vaccine: MenACWY UK 	<ul style="list-style-type: none"> Primary endpoint: efficacy and safety Secondary endpoints: safety, tolerability, reactogenicity, and immunogenicity 	<ul style="list-style-type: none"> FPCD: Q2 2020 LPCD: Q4 2020
Phase II/III (UK, Brazil, South Africa, Poland) NCT04973449	Healthy adults Aged > 18 years	2590	<ul style="list-style-type: none"> Partially double-blinded, randomized, active-controlled trial in unvaccinated or previously vaccinated participants AZD2816 Vaxzevria 	<ul style="list-style-type: none"> Primary endpoint: safety, tolerability Secondary endpoint: immunogenicity 	<ul style="list-style-type: none"> FPCD: Q2 2021 Data readout: Q1 2022 Primary endpoint met



Vaxzevria (SARS-CoV-2)

Prevention of COVID-19

Approved medicines
Late-stage development
Early development

Trial	Population	Patients	Design	Endpoints	Status
Phase II COV006 (UK) NCT_ISRCTN15638344 Partnered (Oxford University)	Healthy children and adolescents age 6-17 years	261	<ul style="list-style-type: none"> Single-blinded, randomized multicentre trial Vaxzevria Meningococcal Group B vaccine UK 	<ul style="list-style-type: none"> Primary endpoints: safety, tolerability, reactogenicity Secondary endpoints: immunogenicity 	<ul style="list-style-type: none"> FPCD: Q1 2021 LPCD: Q2 2021
Phase I/II COV001 (UK) NCT04324606 Partnered (Oxford University)	Healthy adults Age 18-55 years	1077	<ul style="list-style-type: none"> Single-blinded, randomised, controlled, multicentre trial Vaxzevria Control vaccine: MenACWY UK 	<ul style="list-style-type: none"> Primary endpoint: efficacy and safety Secondary endpoints: safety, tolerability, reactogenicity, and immunogenicity 	<ul style="list-style-type: none"> FPCD: Q2 2020 LPCD: Q2 2020
Phase I/II COV005 (SA) NCT04444674 Partnered (Oxford University)	Healthy adults Age 18-65 years HIV+ subgroup	2130	<ul style="list-style-type: none"> Adaptive, double-blinded, randomised placebo-controlled trial Vaxzevria placebo South Africa 	<ul style="list-style-type: none"> Primary endpoint: efficacy, safety, and immunogenicity 	<ul style="list-style-type: none"> FPCD: Q2 2020 LPCD: Q4 2020
Phase I/II D8111C00002 (Japan) NCT04568031	Healthy adults Age ≥18 years	256	<ul style="list-style-type: none"> Double-blinded, randomised, placebo-controlled multicentre trial Vaxzevria placebo Japan 	<ul style="list-style-type: none"> Primary endpoints: safety, tolerability, reactogenicity, immunogenicity Secondary endpoints: immunogenicity 	<ul style="list-style-type: none"> FPCD: Q3 2020 LPCD: Q4 2020 Data readout: Q4 2020

Oncology
CVRM
R&I
Other
V&I
Rare Disease



Vaxzevria (SARS-CoV-2)

Prevention of COVID-19

Approved medicines
Late-stage development
Early development

Trial	Population	Patients	Design	Endpoints	Status
Phase I/II COV004 (Kenya) NCT_tbc Partnered (Oxford University)	Healthy adults	400	<ul style="list-style-type: none"> • Double-blinded, randomised, placebo-controlled multicentre trial • <i>Vaxzevria</i> • Control vaccine: rabies • Kenya 	<ul style="list-style-type: none"> • Primary endpoints: safety, tolerability, reactogenicity, immunogenicity • Secondary endpoints: immunogenicity 	<ul style="list-style-type: none"> • FPCD: Q4 2020 • LPCD: Q3 2021
Phase I COV008 (UK) NCT04816019 Partnered (Oxford University)	Healthy adults Age 18-55	54	<ul style="list-style-type: none"> • Open label, randomized, dose-escalation trial of <i>Vaxzevria</i> administered intranasally • <i>Vaxzevria</i> • UK 	<ul style="list-style-type: none"> • Primary endpoint: safety, tolerability • Secondary endpoint: immunogenicity 	<ul style="list-style-type: none"> • FPCD: Q1 2021 • LPCD: Q4 2020

Oncology

CVRM

R&I

Other

V&I

Rare Disease



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 40 kg) 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	200	<ul style="list-style-type: none"> Randomized, 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, UK 	<ul style="list-style-type: none"> Primary: Safety and tolerability; incidence of antidrug antibodies (ADA) Secondary: Individual serum concentration; GMTs and GMFR in severe acute respiratory coronavirus-2 neutralizing antibodies 	<ul style="list-style-type: none"> FPCD: Q2 2022 Data anticipated: H2 2023



Nirsevimab (Respiratory syncytial virus mAb-YTE)

Infection

Trial	Population	Patients	Design	Endpoints	Status
Phase III MELODY NCT03979313	Healthy infants (born 35 weeks 0 days or greater GA)	3000	<ul style="list-style-type: none"> Randomised, Double-blind, placebo-controlled Arm 1: nirsevimab i.m. Arm 2: placebo i.m. Global trial – 31 countries 	<ul style="list-style-type: none"> Primary endpoints: efficacy Secondary endpoints: safety, PK, ADA 	<ul style="list-style-type: none"> FPCD: Q2 2021 (safety cohort) LPCD: Q4 2021 (safety cohort) Data Anticipated: H2 2022 (safety cohort) 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 GA)	800	<ul style="list-style-type: none"> Randomised, Double-blind, placebo-controlled Arm 1: nirsevimab i.m. Arm 2: placebo i.m. China Only 	<ul style="list-style-type: none"> Primary endpoints: efficacy Secondary endpoints: safety, PK, ADA 	<ul style="list-style-type: none"> FPCD: Q4 2021 Data anticipated: >2023
Phase IIb NCT02878330	29-35 WK GA (gestational age) infants	1453	<ul style="list-style-type: none"> Randomised, double-blind, placebo-controlled trial Arm 1: nirsevimab 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 preterm (born 35 weeks 0 day or less GA), CHD and CLD infants eligible to receive <i>Synagis</i>	925	<ul style="list-style-type: none"> Randomised, Double-blind, palivizumab-controlled Arm 1: nirsevimab i.m. Arm 2: <i>Synagis</i> i.m. Global trial – 32 countries 	<ul style="list-style-type: none"> Primary endpoints: safety and tolerability Secondary endpoints: PK, 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 nirsevimab 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, ADA, efficacy 	<ul style="list-style-type: none"> FPCD: Q3 2020 Data anticipated: H1 2023
Phase I China NCT04840849	Healthy Chinese adults, 18-45 years of age	24	<ul style="list-style-type: none"> Randomised, Double-blind, placebo-controlled Arm 1: nirsevimab i.m. Arm 2: placebo i.m. Route of administration: i.m. China only 	<ul style="list-style-type: none"> Primary endpoint: PK Secondary endpoints: ADA, safety 	<ul style="list-style-type: none"> FPCD: Q2 2021 LPCD: Q2 2021 Data readout: Q2 2022



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 endpoint: safety and tolerability	<ul style="list-style-type: none">FPCD: Q3 2022Data anticipated: H2 2023

Oncology

CVRM

R&I

Other

V&I

Rare Disease



AZD2373

Chronic kidney disease

Approved medicines

Late-stage development

Early development

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 cohortArm 1: AZD2373 s.c.Arm 2: placebo s.c.US	<ul style="list-style-type: none">Primary endpoints: safety and tolerabilitySecondary endpoint: PK parameters	<ul style="list-style-type: none">FPCD: Q1 2020LPCD: Q3 2021Data anticipated: H2 2022
Phase I NCT05351047	Healthy Volunteers	40	<ul style="list-style-type: none">MAD. Dose escalation in 3 cohorts with optional additional 2 cohorts.6 volunteers per cohort receiving AZD2373 and 2 volunteers per cohort receiving placebo.Arm 1: AZD2373 s.c.Arm 2: Placebo s.c.US	<ul style="list-style-type: none">Primary endpoints: safety and tolerabilitySecondary endpoints: PK parameters. Effect of SC MAD administrations of AZD2373 on plasma concentrations of APOL1 protein. Determine APOL1 G0, G1, G2 allele genotype status in study participants.	<ul style="list-style-type: none">FPCD: Q2 2022

Oncology

CVRM

R&I

Other

V&I

Rare Disease



AZD2693 (antisense oligonucleotide)

NASH

Trial	Population	Patients	Design	Endpoints	Status
Phase I NCT04142424	Healthy volunteers	62	<ul style="list-style-type: none"> SAD. 6 cohorts with 6 volunteers receiving AZD2693 and 2 volunteers receiving placebo in each cohort Arm 1: AZD2693 s.c. Arm 2: placebo s.c. US 	<ul style="list-style-type: none"> Primary endpoints: safety and tolerability Secondary endpoint: PK 	<ul style="list-style-type: none"> FPCD: Q4 2019 LPCD: Q3 2021 Data readout: Q1 2022
Phase I NCT04483947	NASH/NAFLD F0-F3	60	<ul style="list-style-type: none"> MAD. 3 cohorts receiving AZD2693 and placebo in each cohort Arm 1: AZD2693 s.c. Arm 2: placebo s.c. US 	<ul style="list-style-type: none"> Primary endpoints: safety and tolerability Secondary endpoint: PK 	<ul style="list-style-type: none"> FPCD: Q2 2021 Data anticipated: H1 2023
Phase I NCT05107336	Healthy Participants	22	<ul style="list-style-type: none"> MAD. 2 cohorts receiving AZD2693 and placebo in each cohort Arm 1: AZD2693 s.c. Arm 2: placebo s.c. JP 	<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: H1 2023



AZD3366

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 NCT04588727	Healthy volunteers	103	<ul style="list-style-type: none">• SAD trial• Part A• 7 cohorts with 6 volunteers receiving AZD3366 and 2 volunteers receiving placebo in each cohort; three cohorts with Japanese subjects 5 receiving AZD3366• 1 receiving placebo; 1 Chinese cohort of 6 receiving AZD3366 2 receiving placebo• Arm 1: AZD3366• Arm 2: placebo• Part B• 12 subjects• Arm 1: AZD3366 + <i>Brilinta</i> + ASA• Arm 2: placebo + <i>Brilinta</i> + ASA	<ul style="list-style-type: none">• Primary endpoints: safety and tolerability• Secondary endpoint: PK parameters	<ul style="list-style-type: none">• FPCD: Q4 2020• Data readout: Q3 2022



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 I NCT04630067	SAD – Healthy volunteers MAD – Heart failure	104	<ul style="list-style-type: none">• Multicentre single and multiple ascending dose study• Part A SAD 6 cohorts• Arm 1: AZD3427• Arm 2: placebo• Part B MAD• Arm 1: AZD3427• Arm 2: placebo• US	<ul style="list-style-type: none">• Primary endpoints: safety and tolerability	<ul style="list-style-type: none">• FPCD: Q4 2020• LPCD: Q3 2022• Data anticipated: H2 2022



AZD4831 (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 Patients	14	<ul style="list-style-type: none">• Open label• AZD4831• AZD4831 and Itraconazole• UK	<ul style="list-style-type: none">• Primary endpoints: PK parameters• Secondary endpoints: safety and tolerability	<ul style="list-style-type: none">• FPCD: Q1 2022• LPCD: Q3 2022• Data anticipated: H2 2022



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	SAD – Healthy volunteers MAD – Healthy volunteers	0	<ul style="list-style-type: none">• Single centre single and multiple ascending dose study• Part A SAD 8 cohorts• Arm 1: AZD5462• Arm 2: placebo• Part B MAD 5 cohorts• Arm 1: AZD5462• Arm 2: placebo• US	<ul style="list-style-type: none">• Primary endpoints: safety and tolerability	<ul style="list-style-type: none">• FPCD: Q4 2021• LPCD: Q2 2022• Data anticipated: H2 2022



AZD5718 (FLAP inhibitor)

Cardiovascular disease & Chronic Kidney Disease

Trial	Population	Patients	Design	Endpoints	Status
Phase IIb FLAIR NCT04492722	Proteinuric CKD	692	<ul style="list-style-type: none"> Randomised, double-blind, placebo-controlled, multicentre, dose-ranging trial AZD5718 placebo 	<ul style="list-style-type: none"> Primary endpoints: dose-response efficacy, safety, PK 	<ul style="list-style-type: none"> FPCD: Q4 2020 Data anticipated: H1 2023
Phase IIa NCT03317002	CAD	129	<ul style="list-style-type: none"> Arm 1: AZD5718 Dose A Arm 2: AZD5718 Dose B Arm 3: placebo Global trial – three countries in Europe 	<ul style="list-style-type: none"> Primary endpoint: PD effect of AZD5718 by assessment of u-LTE4 	<ul style="list-style-type: none"> FPCD: Q4 2017 LPCD: Q4 2019 Data readout: Q1 2021
Phase I NCT03948451	Healthy patients	6	<ul style="list-style-type: none"> Open label hADME trial a single oral dose of 14C-AZD5718 UK 	<ul style="list-style-type: none"> Primary endpoint: Mass balance, with routes and rates of elimination of 14C-AZD5718; Metabolite profiling and structural identification; PK and total radioactivity 	<ul style="list-style-type: none"> FPCD: Q2 2019 LPCD: Q2 2019 Data readout: Q4 2019
Phase I NCT04087187	Healthy patients	14	<ul style="list-style-type: none"> BA trial. Open-label, randomised, 3-period, 3-treatment, crossover design AZD5718 	<ul style="list-style-type: none"> Primary endpoints: safety and tolerability, PK 	<ul style="list-style-type: none"> FPCD: Q4 2019 LPCD: Q4 2019 Data readout: Q1 2020
Phase I NCT04210388	Healthy patients	12	<ul style="list-style-type: none"> BA trial. Open-label, randomised, single-dose, combined 2x2 dose and 3x3 dose crossover design in fixed sequence. AZD5718 	<ul style="list-style-type: none"> Primary endpoints: bioavailability, safety and tolerability 	<ul style="list-style-type: none"> FPCD: Q1 2020 LPCD: Q1 2020 Data readout: Q2 2020
Phase I NCT04734275	Healthy patients	16	<ul style="list-style-type: none"> BA trial. Open-label, randomised, single-dose, 3-period, single dose, crossover design AZD5718 	<ul style="list-style-type: none"> Primary endpoints: bioavailability, safety and tolerability 	<ul style="list-style-type: none"> FPCD: Q1 2021 LPCD: Q1 2021 Data readout: Q4 2021



AZD7503 (antisense oligonucleotide)

NASH

Approved medicines
Late-stage development
Early development

Trial	Population	Patients	Design	Endpoints	Status
Phase I NCT05143905	Healthy volunteers	64	<ul style="list-style-type: none">SAD. 6 cohorts with 6 volunteers receiving AZD7503 and 2 volunteers receiving placebo in each cohortArm 1: AZD7503 s.c.Arm 2: placebo s.c.US	<ul style="list-style-type: none">Primary endpoints: safety and tolerabilitySecondary endpoint: PK	<ul style="list-style-type: none">FPCD: Q4 2021Data anticipated: H1 2023

Oncology

CVRM

R&I

Other

V&I

Rare Disease



AZD8233 (PCSK9 inhibitor, subcutaneous)

Dyslipidaemia

Trial	Population	Patients	Design	Endpoints	Status
Phase II SOLANO NCT04964557	Dyslipidaemia	376	<ul style="list-style-type: none"> Arm 1: AZD8233 dose Arm 2: placebo 28 weeks 8 countries (US, Slovakia, Denmark, Poland, Spain, Netherlands, Czech Republic, Hungary) 	<ul style="list-style-type: none"> Primary endpoints: safety and efficacy - effect of AZD8233 vs Pbo on LDL-C from baseline 	<ul style="list-style-type: none"> FPCD: Q3 2021 LPCD: Q4 2021 Data anticipated: H2 2022
Phase II HAYATE NCT04823611	Dyslipidaemia	82	<ul style="list-style-type: none"> Part A: 11 subjects randomized in an 8:3 ratio Arm 1: AZD8233 dose Arm 2: placebo Part B: 60 subjects randomized across three different treatment arms in a 1:1:1 ratio Arm 1: Medium AZD8233 dose Arm 2: Low AZD8233 dose Arm 3: placebo Part C: 11 subjects randomized in an 8:3 ratio Arm 1: Medium AZD8233 dose Arm 2: placebo 12 weeks Japan 	<ul style="list-style-type: none"> Part A primary endpoints: safety and tolerability Part B primary endpoint: efficacy Part C primary endpoints: safety and tolerability 	<ul style="list-style-type: none"> FPCD: Q1 2021 LPCD: Q1 2022 Data anticipated: H2 2022



AZD9977 (MR modulator)

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> 10 mg Arm 2: AZD9977 B + <i>Farxiga</i> 10 mg Arm 3: AZD9977 C + <i>Farxiga</i> 10 mg Arm 4: <i>Farxiga</i> 10 mg 12 weeks Trial conducted in 19 countries globally 	<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: H1 2023
Phase I NCT04469907	Renal impairment	18	<ul style="list-style-type: none"> AZD9977 US 	<ul style="list-style-type: none"> Primary endpoints: PK parameters Secondary endpoints: safety and tolerability 	<ul style="list-style-type: none"> FPCD: Q3 2020 LPCD: Q4 2021 Data readout: Q2 2022
Phase I NCT04686591	Healthy volunteers	8	<ul style="list-style-type: none"> hADME trial single oral dose 14C-AZD9977 UK 	<ul style="list-style-type: none"> Primary endpoints: absolute bioavailability, the mass balance, rates and routes of elimination Secondary endpoints: safety and tolerability 	<ul style="list-style-type: none"> FPCD: Q1 2021 LPCD: Q1 2021 Data readout: Q1 2022
Phase I NCT04798222	Healthy volunteers	20	<ul style="list-style-type: none"> Rel BA trial Study to Assess the Bioavailability of Different Formulations of AZD9977 and Dapagliflozin and Influence of Food in Selected Formulations Germany 	<ul style="list-style-type: none"> Primary endpoints: relative bioavailability of 9977 and Dapagliflozin in different capsule formulations; PK parameters 	<ul style="list-style-type: none"> FPCD: Q3 2021 LPCD: Q3 2021 Data readout: Q1 2022



Cotadutide (GLP-1-glucagon agonist)

Diabetes/CKD, NASH

Trial	Population	Patients	Design	Endpoints	Status
Phase II NCT04019561	Obese patients with non-alcoholic fatty liver disease (NAFLD)/non-alcoholic steatohepatitis (NASH)	74	<ul style="list-style-type: none"> Arm 1: cotadutide high dose s.c. Arm 2: placebo high dose s.c. Arm 3: cotadutide low dose s.c. Arm 4: placebo low dose s.c. US 	<ul style="list-style-type: none"> Primary endpoints: safety and tolerability Secondary endpoints: change in hepatic fat fraction; change in liver fat volume; change in visceral adipose tissue 	<ul style="list-style-type: none"> FPCD: Q4 2019 LPCD: Q2 2021 Data readout: Q1 2022
Phase II NCT04515849	Chronic kidney disease with type 2 diabetes mellitus	225	<ul style="list-style-type: none"> Arm 1: cotadutide 100 micrograms Arm 2: cotadutide 300 micrograms Arm 3: cotadutide 600 micrograms Arm 4: semaglutide Arm 5: placebo 	<ul style="list-style-type: none"> Primary endpoint: efficacy change in UACR Secondary endpoints: Change in HbA1c; change in glucose measured by CGM; effects on body weight; safety, tolerability, Immunogenicity 	<ul style="list-style-type: none"> FPCD: Q4 2020 LPCD: Q3 2021 Data readout: Q2 2022



MEDI6570

Cardiovascular

Trial	Population	Patients	Design	Endpoints	Status
Phase IIb NCT04610892	Post MI	400	<ul style="list-style-type: none"> Evaluation of anti-inflammatory potential and effect on surrogates for atherosclerotic and heart failure (HF) events Arm 1: High MEDI6570 dose Arm 2: Medium MEDI6570 dose Arm 3: Low MEDI6570 dose Arm 4: placebo US, Canada, Hungary, Japan, Czech Republic, Italy, Spain, Netherlands, Poland, UK, Australia, Russia 	<ul style="list-style-type: none"> Primary endpoints: efficacy and safety 	<ul style="list-style-type: none"> FPCD: Q4 2020 Data anticipated: H2 2023



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 CKD	70	<ul style="list-style-type: none">• SAD trial• 6 cohorts• Arm 1: MEDI8367 s.c.• Arm 2: placebo s.c.• US	<ul style="list-style-type: none">• Primary endpoints: safety and tolerability• Secondary endpoints: PK parameters, ADA	<ul style="list-style-type: none">• FPCD: Q3 2020• LPCD: Q4 2020• Data readout: Q2 2022



Zibotentan (endothelin receptor antagonist)

Chronic kidney disease

Trial	Population	Patients	Design	Endpoints	Status
Phase IIb ZENITH-CKD NCT04724837	CKD	495	<ul style="list-style-type: none"> • Arm 1: zibotentan dose A + <i>Farxiga</i> 10 mg once daily • Arm 2: zibotentan dose B + <i>Farxiga</i> 10 mg once daily • Arm 3: <i>Farxiga</i> 10 mg + placebo once daily • • 	<ul style="list-style-type: none"> • Primary endpoint: • Change in log-transformed UACR from baseline to week 12. Zibotentan Dose B/dapagliflozin 10 mg versus dapagliflozin 10 mg. • • Secondary endpoints: • Change in Log-transformed UACR from baseline to Week 12. Zibotentan dose A/dapagliflozin 10 mg versus dapagliflozin 10 mg monotherapy. • 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 anticipated: H1 2023
Phase I NCT04991571	Healthy volunteers	28	<ul style="list-style-type: none"> • Part 1 of the study is intended to collect samples for Metabolites in Safety Testing (MIST) analysis after administration of multiple doses of zibotentan. • Part 2 of the study is designed to evaluate the relative bioavailability of zibotentan and dapagliflozin after dosing with two different fixed-dose combination (FDC) formulations and dosing with separate formulations of zibotentan and dapagliflozin 	<ul style="list-style-type: none"> • Part 1: • Metabolites in Safety Testing MIST analysis. No PK statistical analysis will be performed. • Part 2: • PK parameters 	<ul style="list-style-type: none"> • FPCD: Q3 2021 • LPCD: Q4 2021 • Data anticipated: H2 2022



Zibotentan (endothelin receptor antagonist)

Liver Cirrhosis with Features of portal hypertension

Trial	Population	Patients	Design	Endpoints	Status
Phase I NCT05112419	Cohort 1: Moderate hepatic impairment/moderate renal impairment patients Cohort 2: Healthy volunteers	24	<ul style="list-style-type: none"> Single dose, single centre, Open label, Parallel group study to investigate the pharmacokinetics, safety and tolerability of 5 mg Zibotentan 	<ul style="list-style-type: none"> Primary Endpoints: Area under plasma concentration-time curve from time zero to infinity (AUC_{inf}) Area under the plasma concentration-curve from time zero to time of last quantifiable concentration (AUC_{last}) Maximum observed plasma concentration (C_{max}) 	<ul style="list-style-type: none"> FPCD: Q4 2021 LPCD: Q4 2021 Data anticipated: H2 2022



AZD1402 (IL-4 receptor alpha antagonist)

Asthma

Trial	Population	Patients	Design	Endpoints	Status
Phase IIa Apatura NCT04643158	Patients with asthma on medium dose inhaled corticosteroids	345	<ul style="list-style-type: none"> Randomised, placebo-controlled, double-blinded, multicentre, 2-part trial. Part 1 population with asthma controlled on medium dose ICS-LABA <ul style="list-style-type: none"> Part 1a <ul style="list-style-type: none"> Arm 1: AZD1402 Dose 1 (low) (DPI) Arm 2: AZD1402 Dose 2 (DPI) Arm 3: placebo Part 1b <ul style="list-style-type: none"> Arm 1: AZD1402 Dose 3 (high) (DPI) Arm 2: placebo Part 2 population uncontrolled on medium dose ICS-LABA <ul style="list-style-type: none"> Arm 1: AZD1402 Dose 1 (DPI) Arm 2: AZD1402 Dose 2 (DPI) Arm 3: AZD1402 Dose 3 (DPI) Arm 4: placebo Ukraine, Australia, Germany, Hungary, Korea, Poland, Spain, UK 	<ul style="list-style-type: none"> Part 1 primary endpoints: safety and tolerability, PK Part 2 primary endpoint: change in FEV1 	<ul style="list-style-type: none"> FPCD: Q2 2021 Data anticipated: H2 2022



AZD4604 (inhaled JAK-1 inhibitor)

Asthma

Approved medicines

Late-stage development

Early development

Trial	Population	Patients	Design	Endpoints	Status
Phase I NCT04769869	Healthy subjects 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	<ul style="list-style-type: none">• Primary endpoints: safety and tolerability• Secondary endpoints: PK parameters, FENO	<ul style="list-style-type: none">• FPCD: Q4 2021• Data anticipated: H1 2023

Oncology

CVRM

R&I

Other

V&I

Rare Disease



AZD5055 (oral porcupine inhibitor)

Idiopathic pulmonary fibrosis (IPF) and other ILDs with progressive fibrosis

Trial	Population	Patients	Design	Endpoints	Status
Phase I NCT05134727	Healthy subjects	90	<ul style="list-style-type: none"> SAD/MAD trial Part 1a (fasted state) and 1b (fed state) 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 Data anticipated: H1 2023



AZD5718 (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	1928	<ul style="list-style-type: none">• Randomised, placebo-controlled, double-blind, multicentre, 2-part study with an active comparator (montelukast) arm, and a lead-in pharmacokinetics (PK) cohort.• PK cohort• Arm 1: AZD5718• Arm 2: placebo• Part 1• Arm 1: AZD5718• Arm 2: placebo• Part 2• Arm 1: AZD5718 Dose A• Arm 2: AZD5718 Dose B• Arm 3: AZD5718 Dose C• Arm 4: montelukast• Arm 5: placebo• US, Hungary, Japan, Netherlands, Poland	<ul style="list-style-type: none">• Primary endpoint; Time to first CompEx Asthma event (Composite endpoint for Exacerbations)	<ul style="list-style-type: none">• FPCD: Q2 2022• Data anticipated: >2023



AZD8630 (inhaled TSLP)

Asthma

Approved medicines

Late-stage development

Early development

Trial	Population	Patients	Design	Endpoints	Status
Phase I NCT05110976 Partnered (AMGEN)	Healthy subjects and patients with asthma	232	<ul style="list-style-type: none">SAD & MAD trial	<ul style="list-style-type: none">Primary endpoints: safety and tolerabilitySecondary endpoints: PK parameters, FENO	<ul style="list-style-type: none">FPCD: Q1 2022Data anticipated: H2 2023

Oncology

CVRM

R&I

Other

V&I

Rare Disease



AZD4041 (orexin 1 receptor antagonist)

Opioid use disorder

Trial	Population	Patients	Design	Endpoints	Status
Phase I NCT04076540 Partnered with Eolas Therapeutics Inc and NIH (Partnered with Eolas Therapeutics Inc and NIH)	Healthy volunteers	48	<ul style="list-style-type: none"> • Randomised, double blind, SAD trial • Arm 1: AZD4041 • Arm 2: placebo • US only 	<ul style="list-style-type: none"> • Primary endpoints: safety and tolerability • Secondary endpoints: PK, PD 	<ul style="list-style-type: none"> • FPCD: Q4 2019 • Data readout: Q4 2021 • Primary endpoint met
Phase I NCT05209334 Partnered with Eolas Therapeutics Inc and NIH (Partnered with Eolas Therapeutics Inc and NIH)	Healthy volunteers	36	<ul style="list-style-type: none"> • Randomised, double blind MAD trial • Arm 1: AZD4041 • Arm 2: placebo • Canada only 	<ul style="list-style-type: none"> • Primary Endpoints: safety and tolerability • Secondary endpoint: PK 	<ul style="list-style-type: none"> • FPCD: Q1 2022 • LPCD: Q2 2022 • Data anticipated: H2 2022



MEDI0618 (PAR2 antagonist mAb)

Osteoarthritis pain

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	<ul style="list-style-type: none">• FPCD: Q4 2019• Data readout: Q2 2022



MEDI1341 (alpha-synuclein mAb)

Parkinson's disease

Trial	Population	Patients	Design	Endpoints	Status
Phase I NCT03272165 Partnered (Takeda)	Healthy volunteers	48	<ul style="list-style-type: none"> SAD 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, PD 	<ul style="list-style-type: none"> FPCD: Q4 2017 LPCD: Q4 2020 Data readout: Q4 2021
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, PD 	<ul style="list-style-type: none"> FPCD: Q3 2020 LPCD: Q3 2021 Data anticipated: H2 2022



MEDI7352 (NGF TNF bispecific mAb)

Osteoarthritis pain

Trial	Population	Patients	Design	Endpoints	Status
Phase IIb NCT04675034	Painful osteoarthritis of the knee	300	<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, PD, ADA 	<ul style="list-style-type: none"> FPCD: Q1 2021 Data anticipated: H2 2023
Phase IIa NCT03755934	Painful diabetic neuropathy	271	<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, PD 	<ul style="list-style-type: none"> FPCD: Q4 2018 Data anticipated: >2023
Phase I NCT02508155	Painful osteoarthritis of the knee	160	<ul style="list-style-type: none"> SAD & MAD trial Arm 1: MEDI7352 i.v. Arm 2: placebo i.v. Arm 3: MEDI7352 s.c Arm 4: placebo s.c Europe only 	<ul style="list-style-type: none"> Primary endpoints: safety and tolerability Secondary endpoints: PK, PD 	<ul style="list-style-type: none"> FPCD: Q1 2016 LPCD: Q4 2020 Data readout: Q2 2021
Phase I NCT04770428	Healthy volunteers Japanese and Caucasian	20	<ul style="list-style-type: none"> MAD trial Arm 1: MEDI7352 s.c Arm 2: placebo s.c Europe only 	<ul style="list-style-type: none"> Primary endpoints: safety and tolerability Secondary endpoints: PK, PD, ADA 	<ul style="list-style-type: none"> FPCD: Q2 2021 LPCD: Q3 2021 Data readout: Q4 2021



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



Koselugo (selumetinib, MEK inhibitor)

Neurofibromatosis Type 1 (NF1)

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"> Multicentre, international study with a parallel, randomized, double blind, placebo controlled, 2 arm design Arm A: <i>Koselugo</i> 25mg/m² BID Arm B: Placebo BID until end of Cycle 12, then cross over 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. Key secondary endpoint: Chronic PN-pain intensity (change from baseline) on <i>Koselugo</i> vs placebo 	<ul style="list-style-type: none"> FPCD: Q4 2021 Data anticipated: >2023



Koselugo (selumetinib, MEK inhibitor)

Paediatric neurofibromatosis type 1, solid tumours

Trial	Population	Patients	Design	Endpoints	Status
Phase II SPRINT NCT01362803 Partnered (NCI)	Paediatric NF1	75	<ul style="list-style-type: none"> Single arm: <i>Koselugo</i> 25mg/m² BID with 2 strata: Stratum 1: PN related morbidity present at enrolment Stratum 2: no PN related morbidity present at enrolment 	<ul style="list-style-type: none"> Primary endpoint: Complete partial and complete response rate measured by volumetric MRI, Duration of response and functional outcomes/QoL 	<ul style="list-style-type: none"> FPCD: Q3 2015 LPCD: Q4 2016 Data readout: Q1 2019 Primary endpoint met
Phase I/II SPRINKLE NCT05309668	Child age 1-6 years diagnosed with NF1 with symptomatic, inoperable PN Must have at least one measurable PN, defined as a PN of at least 3 cm measured in one dimension	38	<ul style="list-style-type: none"> Single-Arm - Selumetinib Open Label 	<ul style="list-style-type: none"> Primary Endpoints - Selumetinib 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 study treatment (Cycle 1 Day 1) (each cycle is 28 days)] - Adverse Events 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: H2 2023
Phase I Japan PK / Safety trial NCT04495127	Paediatric inoperable NF1-PN patients	44904	<ul style="list-style-type: none"> Open-label trial <i>Koselugo</i> in Japanese paediatric NF1-PN patients 	<ul style="list-style-type: none"> Primary endpoint: safety Secondary endpoints: PK, anti-tumour effect 	<ul style="list-style-type: none"> FPCD: Q3 2020 LPCD: Q4 2020 Data readout: Q4 2021
Phase I China PK / Safety / Efficacy trial NCT04590235	Pediatric (2-17 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 (24mths post LSD) Long term follow up (60mths post LSD) 	<ul style="list-style-type: none"> Primary endpoints: safety, tolerability and PK Secondary endpoints: efficacy (ORR, DoR; TTR; PFS) 	<ul style="list-style-type: none"> FPCD: Q1 2021 Data anticipated: H2 2023
Phase I 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	20	<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 versus the same dose given in a fasted state. 	<ul style="list-style-type: none"> Primary endpoints: PK (steady state systemic exposure), safety (especially GI toxicity) 	<ul style="list-style-type: none"> FPCD: Q3 2021 Data anticipated: H1 2023



Soliris (anti-complement C5 mAb)

Neurology

Approved medicines

Late-stage development

Early development

Trial	Population	Patients	Design	Endpoints	Status
Phase III ECU-GBS-301 NCT04752566	Guillain Barré syndrome	57	<ul style="list-style-type: none">• Arm 1: <i>Soliris</i> i.v. once weekly for 4 weeks• Arm 2: Placebo• Japan-only	<ul style="list-style-type: none">• Primary endpoint: Time to first reaching a Hughes functional grading scale score ≤ 1	<ul style="list-style-type: none">• FPCD: Q1 2021• Data anticipated: H2 2022

Oncology

CVRM

R&I

Other

V&I

Rare Disease



Ultomiris (anti-complement C5 mAb)

Haematology & nephrology

Trial	Population	Patients	Design	Endpoints	Status
Phase III ALXN-1210-PNH-303 NCT03748823	PNH and aHUS	136	<ul style="list-style-type: none"> • <i>Ultomiris</i> s.c. 	<ul style="list-style-type: none"> • Primary endpoint: Day 71 serum <i>Ultomiris</i> C trough 	<ul style="list-style-type: none"> • FPCD: Q1 2019 • Data readout: Q2 2020 • Primary endpoint met
Phase III ALXN-1210-TMA-315 NCT04743804	Thrombotic microangiopathy associated with a trigger	100	<ul style="list-style-type: none"> • Arm 1: <i>Ultomiris</i> Q8W • Arm 2: Placebo 	<ul style="list-style-type: none"> • Primary endpoint: Complete TMA response • Secondary endpoints: Time to complete TMA response, Hematologic response, Renal response 	<ul style="list-style-type: none"> • FPCD: Q3 2021 • Data anticipated: >2023
Phase III ALXN-1210-TM-313 NCT04543591	Thrombotic microangiopathy associated with haematopoietic stem cell transplant	184	<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: >2023
Phase III ALXN-1210-TM-314 NCT04557735	Paediatric thrombotic microangiopathy associated with haematopoietic stem cell transplant	40	<ul style="list-style-type: none"> • Arm 1: <i>Ultomiris</i> administered once every 4-8 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: >2023
Phase II ALXN-1210-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: Both cohorts, percentage change in proteinuria from baseline to Week 26 • Secondary endpoints: Both cohorts, percentage change in proteinuria from baseline to Week 50 	<ul style="list-style-type: none"> • FPCD: Q1 2021 • Data anticipated: >2023



Ultomiris (anti-complement C5 mAb)

Neurology

Approved medicines

Late-stage development

Early development

Trial	Population	Patients	Design	Endpoints	Status
Phase III ALXN-1210-NMO-307 NCT04201262	Neuromyelitis optica spectrum disorder	58	<ul style="list-style-type: none"> Arm 1: <i>Ultomiris</i> Q8W 	<ul style="list-style-type: none"> Primary endpoint: time to first adjudicated on-trial relapse 	<ul style="list-style-type: none"> FPCD: Q4 2019 LPCD: Q1 2021 Data readout: Q2 2022 Primary endpoint met
Phase III ALXN-1210-MG-306 NCT03920293	Generalised myasthenia gravis	175	<ul style="list-style-type: none"> Arm 1: <i>Ultomiris</i> 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"> Data readout: Q2 2021 Primary endpoint met
Phase II/III ALXN-1210-ALS-308 NCT04248465	Amyotrophic lateral sclerosis	382	<ul style="list-style-type: none"> Arm 1: <i>Ultomiris</i> Arm 2: placebo 	<ul style="list-style-type: none"> Primary endpoint: change from baseline in ALSFRS-R total score 	<ul style="list-style-type: none"> Trial halted post futility analysis Q3 2021
Phase II/III ALXN1210-DM-310 NCT04999020	Dermatomyositis	180	<ul style="list-style-type: none"> Arm 1: <i>Ultomiris</i> Arm 2: placebo 	<ul style="list-style-type: none"> Primary endpoint: improvement response on International Myositis Assessment And Clinical Studies-Total Improvement Score (IMACS-TIS) 	<ul style="list-style-type: none"> FPCD: Q4 2021 Data anticipated: >2023

Oncology

CVRM

R&I

Other

V&I

Rare Disease



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: 800 mg acoramidis (ALXN2060) administered twice daily	<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, All-cause mortality and cardiovascular-related hospitalization over a 30-month period	<ul style="list-style-type: none">FPCD: Q4 2020Data anticipated: >2023

Oncology

CVRM

R&I

Other

V&I

Rare Disease



ALXN1840 (bis-choline tetrathiomolybdate)

Wilson disease

Trial	Population	Patients	Design	Endpoints	Status
Phase III WTX101-301 FoCus NCT03403205	Wilson Disease	215	<ul style="list-style-type: none"> Arm 1: ALXN1840 was administered orally for 48 weeks at doses ranging from 15 milligrams (mg) every other day (QOD) up to a titrated dose of 60 mg daily. Arm 2: Standard of care 	<ul style="list-style-type: none"> Primary endpoint: daily mean AUEC of dNCC Secondary endpoint: change from baseline in the UWDRS Part II total score 	<ul style="list-style-type: none"> Data readout: Q3 2021 Primary endpoint met
Phase II ALXN1840-WD-204 NCT04573309	Wilson Disease	10	<ul style="list-style-type: none"> Arm 1: Participants will be administered ALXN1840 at a dose of 15 milligrams (mg)/day on Day 1 through Day 28 and then increased to 30 mg/day on Day 29 through Day 39 	<ul style="list-style-type: none"> Primary endpoint: mean daily copper balance 	<ul style="list-style-type: none"> FPCD: Q3 2020 Data anticipated: H2 2022
Phase II ALXN1840-WD-205 NCT04422431	Wilson Disease	31	<ul style="list-style-type: none"> Arm 1: ALXN1840, Participants will be initiated at 15 milligrams once daily, then the dose will be increased to 30 milligrams once daily at Week 6. 	<ul style="list-style-type: none"> Primary endpoint: change from baseline at Week 48 in liver copper concentration 	<ul style="list-style-type: none"> FPCD: Q4 2020 Data anticipated: H2 2022



ALXN2050 (factor D inhibitor)

Haematology, nephrology, neurology

Approved medicines

Late-stage development

Early development

Trial	Population	Patients	Design	Endpoints	Status
Phase II ALXN2050-gMG-201 NCT05218096	Generalized myasthenia gravis (gMG)	70	<ul style="list-style-type: none">• Arm 1: ALXN2050 180mg• Arm 2: ALXN2050 120mg• Arm 3: placebo followed by ALXN2050	<ul style="list-style-type: none">• Primary endpoint: Myasthenia Gravis Activities of Daily Living (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: >2023

Oncology

CVRM

R&I

Other

V&I

Rare Disease



CAEL-101 (fibril-reactive mAb)

AL amyloidosis

Trial	Population	Patients	Design	Endpoints	Status
Phase III CAEL101-302 NCT04512235	Mayo stage IIIa amyloidosis	267	<ul style="list-style-type: none"> Arm 1: CAEL-101 combined with SoC for plasma cell dyscrasia (PCD) Arm 2: placebo combined with SoC for PCD 	<ul style="list-style-type: none"> Primary: time from first dose of trial drug until death or end of trial Secondary: change in distance walked during a six-minute walk test, and quality of life measures 	<ul style="list-style-type: none"> FPCD: Q4 2020 Data anticipated: >2023
Phase III CAEL101-301 NCT04504825	Mayo stage IIIb amyloidosis	110	<ul style="list-style-type: none"> Arm 1: CAEL-101 combined with SoC for PCD Arm 2: placebo combined with SoC for PCD 	<ul style="list-style-type: none"> Primary: time from first dose of trial drug until death or end of trial Secondary: change in distance walked during a six-minute walk test, and quality of life measures 	<ul style="list-style-type: none"> FPCD: Q1 2021 Data anticipated: >2023
Phase II CAEL101-203 NCT04304144	Mayo Stage I, Stage II and Stage IIIa amyloidosis	25	<ul style="list-style-type: none"> Arm 1: CAEL-101 combined with SoC CyBorD Arm 2: placebo combined with SoC CyBorD and daratumumab 	<ul style="list-style-type: none"> Primary: dose toxicity – occurrence of dose limiting toxicity (DLT) during the first 4 weeks of therapy Secondary: area under the plasma curve concentration versus time curve (AUC) 	<ul style="list-style-type: none"> FPCD: Q1 2020 Data anticipated: >2023



danicopan (ALXN2040 Factor D inhibitor)

Haematology & ophthalmology

Trial	Population	Patients	Design	Endpoints	Status
Phase III ALXN2040-PNH-301 NCT04469465	PNH with clinically relevant extravascular haemolysis	84	<ul style="list-style-type: none"> Arm 1: danicopan (ALXN2040) + 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 anticipated: H1 2023
Phase II ALXN2040-GA-201 NCT05019521	Geographic atrophy	330	<ul style="list-style-type: none"> Arms 1-3: danicopan (ALXN2040) dosed at 100-400mg daily 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: >2023



**Rare Disease –
early-stage development**



ALXN1720 (anti-C5 bi-specific minibody)

Neurology

Approved medicines

Late-stage development

Early development

Trial	Population	Patients	Design	Endpoints	Status
Phase I ALXN1720-HV-101 NCT04920370	Healthy Volunteers	96	<ul style="list-style-type: none">• Single & Multiple Ascending Dosing of ALXN1720 via s.c. and i.v. administration• Placebo	<ul style="list-style-type: none">• Primary: Incidence of TEAEs and SAEs	<ul style="list-style-type: none">• FPCD: Q3 2019• Data readout: Q2 2022

Oncology

CVRM

R&I

Other

V&I

Rare Disease



ALXN1820 (anti-properdin)

Haematology

Approved medicines

Late-stage development

Early development

Trial	Population	Patients	Design	Endpoints	Status
Phase I ALXN1820-HV-101 NCT04631562	Healthy Volunteers	80	<ul style="list-style-type: none">• Arm 1: ALXN1820 administered s.c. or i.v., multiple ascending doses• Arm 2: Placebo	<ul style="list-style-type: none">• Primary: Participants with TEAEs	<ul style="list-style-type: none">• FPCD: Q1 2021• Data anticipated: H1 2023

Oncology

CVRM

R&I

Other

V&I

Rare Disease



ALXN1850 (next generation asfotase alfa)

Hypophosphatasia

Approved medicines
Late-stage development
Early development

Trial	Population	Patients	Design	Endpoints	Status
Phase I ALXN1850-HPP-101 NCT04980248	HPP	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: Incidence of TEAEs and TSEAEs	<ul style="list-style-type: none">FPCD: Q3 2021Data anticipated: H2 2022

Oncology

CVRM

R&I

Other

V&I

Rare Disease



ALXN1910 (Next Gen TNSALP ERT)

Bone metabolism

Approved medicines

Late-stage development

Early development

Oncology

CVRM

R&I

Other

V&I

Rare Disease

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 2022Data anticipated: H1 2023



ALXN2050 (factor D inhibitor)

Haematology, nephrology, neurology

Approved medicines

Late-stage development

Early development

Trial	Population	Patients	Design	Endpoints	Status
Phase II ACH228-110 NCT04170023	PNH	26	<ul style="list-style-type: none">Arm 1: ALXN2050 Monotherapy, with groups including treatment-naïve, C5 inhibitor treatment experienced, and patients previously receiving danicopan (ALXN2040)	<ul style="list-style-type: none">Primary endpoint: Change in haemoglobin relative to baselineSecondary endpoints: Number of participants who have transfusion avoidance, Change in lactate dehydrogenase relative to baseline	<ul style="list-style-type: none">FPCD: Q4 2019Data anticipated: >2023

Oncology

CVRM

R&I

Other

V&I

Rare Disease



List of abbreviations

14C	Radioactive isotope of carbon, Carbon 14
1L, 2L, 3L	1st, 2nd or 3rd line
5-FU	5-fluorouracil
A2AR	Adenosine A2A receptor
ACQ	Asthma control questionnaire
ACR	American college of rheumatology response scoring system
ADA	Anti-drug antibodies
ADC	Antibody-drug conjugate
ADP	Adenosine diphosphate
AE	Adverse event
aHUS	Atypical haemolytic uraemic syndrome
AI	Auto-injector
AKT	Protein kinase B
ALK	Anaplastic large-cell lymphoma kinase
ALSFRS-R	Amyotrophic Lateral Sclerosis Functional Rating Scale-Revised
APFS	Accessorised pre-filled syringe
AQLQ	Asthma quality of life questionnaire
AS	Albuterol sulphate
ATM	Ataxia-telangiectasia mutated kinase
ATR	Ataxia telangiectasia and rad3-related protein
ATTR-CM	Transthyretin amyloid cardiomyopathy
AUC	Area under curve
AUEC	Area under the effect-time curve
B7RP	B7-related protein-1
BA	Bioavailability
BAFF	B-cell activating factor
BCG	Bacillus Calmette–Guérin
BCMA	B-cell maturation antigen
BDA	Budesonide albuterol
BFF	Budesonide and formoterol fumarate
BGF	Budesonide, glycopyrronium and formoterol fumarate
BICR	Blinded independent central review
BID	Bis in die (twice per day)
BIG	Big ten cancer research consortium
BMD	Bone mineral density
BMFI	Bone metastasis-free interval
BMI	Body mass index
BRCAwt	Breast cancer wild-type gene
BRD4	Bromodomain-containing protein 4
BTC	Biliary tract carcinoma

BTK	Bruton's tyrosine kinase
CA-125	Cancer antigen 125
CAD	Coronary artery disease
CBR	Clinical benefit rate
CD	Cluster of differentiation
CDK	Cyclin-dependent kinase
CE	Clinically evaluable
CHD	Coronary heart disease
Chemo	Chemotherapy
CHF	Chronic heart failure
CKD	Chronic kidney disease
CLL	Chronic lymphocytic leukaemia
CMAx	Maximum observed plasma concentration
C-MET	Tyrosine-protein kinase Met
CNS	Central nervous system
COPD	Chronic obstructive pulmonary disease
CR	Complete response
CRC	Colorectal cancer
CrCl	Creatinine clearance
CRR	Complete response rate
CTC	Circulating tumour cell
CTLA-4	Cytotoxic T-lymphocyte-associated antigen 4
CV	Cardiovascular
CVOT	Cardiovascular outcomes trial
CVRM	Cardiovascular renal and metabolism
CXCR2	C-X-C Motif chemokine receptor 2
DB	Double blind
DC	Disease control
DCR	Disease control rate
DDI	Drug-drug Interaction
dECG	Differentiated electrocardiogram
DFS	Disease free survival
DLBCL	Diffuse large B-cell lymphoma
DLT	Dose-limiting toxicity
DMARDs	Disease-modifying antirheumatic drugs
DNA	Deoxyribonucleic acid
DoCR	Durability of complete response
dNCC	Directly Measured Non-ceruloplasmin-bound Copper
DoR	Duration of response
DPI	Dry powder inhaler

DRFI	Disease recurrence-free interval
DXA	Dual energy X-ray absorptiometry
EBRT	External beam radiation therapy
ECG	Electrocardiogram
EFS	Event-free survival
eGFR	Estimated glomerular filtration rate
EGFR	Epidermal growth factor receptor
ER	Oestrogen receptor
ERK	Extracellular signal-regulated kinase
ESCC	Esophageal squamous cell carcinoma
ESR	Externally sponsored trial
ESR1	Oestrogen receptor 1
ET	Endocrine therapy
FAF	Fundus Autofluorescence
FDC	Fixed-dose combination
FeNO	Fractional nitric oxide concentration in exhaled breath
FEV	Forced-expiratory volume
FGFR	Fibroblast growth factor receptor
FLAP	5-lipoxygenase-activating protein
FPDC	First patient commenced dosing
FPG	Fasting plasma glucose
GA	Gestational age
GA	Geographic atrophy
GBM	Glioblastoma
gBRCAm or tBRCAm	Germline or tumour (somatic) BRCA mutation
GEJ	Gastric/gastro-oesophageal junction
GFF	Glycopyrronium and formoterol fumarate
GLP-1	Glucagon-like peptide-1
GMFRs	Geometric mean fold rises
GMTs	Geometric mean titers
hADME	Human mass balance
HAI	Haemagglutination-inhibition
HbA1c	Haemoglobin A1c
HCC	Hepatocellular carcinoma
HD	High dose
HDL-C	High-density lipoprotein cholesterol
HER2	Human epidermal growth factor receptor 2
HF	Heart failure
HFpEF	Heart failure with preserved ejection fraction



List of abbreviations

HFrEF	Heart failure with reduced ejection fraction
HGFR	Met/hepatocyte growth factor receptor
HGSC	High grade serous carcinoma
hHF	Hospitalisation for heart failure
HIF-PHI	Hypoxia inducible factor - prolyl hydroxylase inhibitor
HNSCC	Head and neck squamous-cell carcinoma
HPV	Human papillomavirus
HRD	Homologous recombination deficiency
HRRm	Homologous recombination repair mutation
i	inhibitor
IA	Investigator-assessed
ICS	Inhaled corticosteroid
ICU	Intensive care unit
IDFS	Invasive disease-free survival
IgAN	Immunoglobulin A nephropathy
IL	Interleukin
i.m.	Intramuscular
IRC	Independent review committee
ISS	Investigator-sponsored studies
i.v.	Intravenous
J-SD	Japanese single dose
Ki67	Protein that is encoded by the MKI67 gene in human
LAAB	Long-acting antibody
LABA	Long -acting beta agonist
LAMA	Long-acting muscarinic agonist
LCAT	Lecithin-cholesterol acyltransferase
LCM	Lifecycle management
LDH	Lactate dehydrogenase
LN	Lupus nephritis
LOCS III	Lens opacities classification system III
LPCD	Last patient commenced dosing
LV	Left ventricle
m	Mutation
mAb	Monoclonal antibody
MABA	Muscarinic antagonist-beta2 agonist
MACE	Major adverse cardiac events
MAD	Multiple ascending dose
MCC	Mucociliary clearance
MCL	Mantle cell lymphoma
MCL1	Myeloid leukemia cell differentiation protein 1
mCRPC	Metastatic castrate-resistant prostate cancer
MD	Medium dose
MDI	Metered-dose inhaler
MDS	Myelodysplastic syndrome
MEK	Mitogen-activated protein kinase
MET	Tyrosine-protein kinase Met
MG-ADL	Myasthenia Gravis-Activities Of Daily Living
MI	Myocardial infarction
MMT	Mixed meal test
MPO	Myeloperoxidase
mPR	Major pathological response
MRI	Magnetic resonance imaging
MTD	Maximum tolerated dose
NaC	Sodium channel
NCI	National cancer institute (US)
NCPV	Noncalcified plaque volume
NF1	Neurofibromatosis type 1
NGF	Nerve growth factor
NHL	Non-Hodgkin's lymphoma
NIH	National Institute of Health (US)
NKG2a	Natural killer cell C-type lectin receptor G2A
NME	New molecular entity
NRG	National clinical trials network in oncology (US)
NSCLC	Non-small cell lung cancer
OCS	Oral corticosteroid
OD	Once daily
OGTT	Oral glucose tolerance test
OR	Objective response
ORR	Objective response rate
OS	Overall survival
PARP	Poly ADP ribose polymerase
PASI	Psoriasis area severity index
PBD	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

