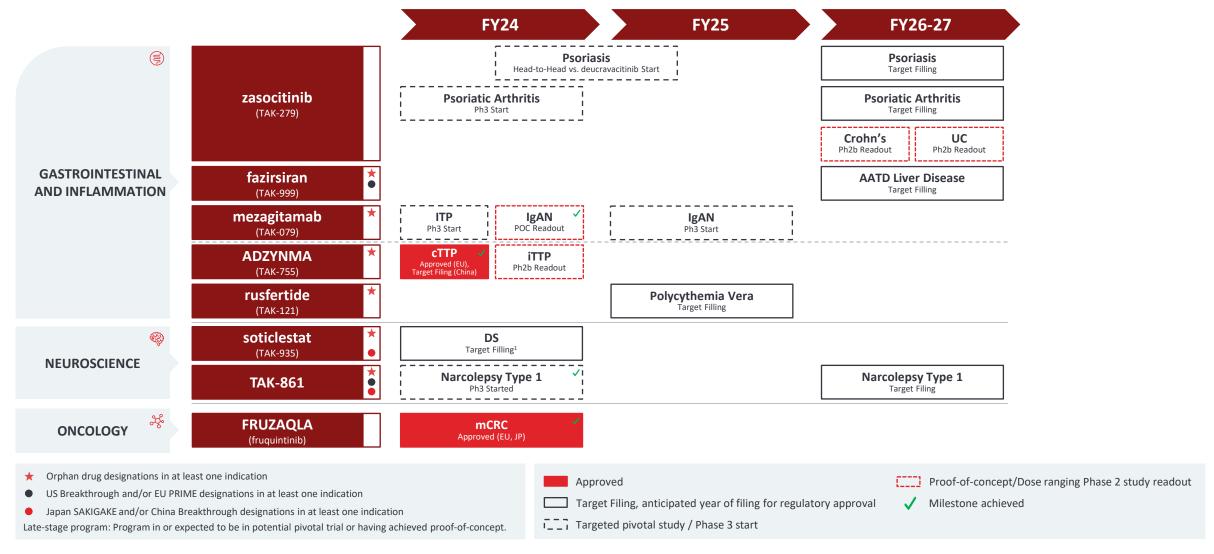
Developing Life Transforming Medicines for Rare and More Prevalent Diseases: Late-Stage Programs have the Potential to Generate Significant Value

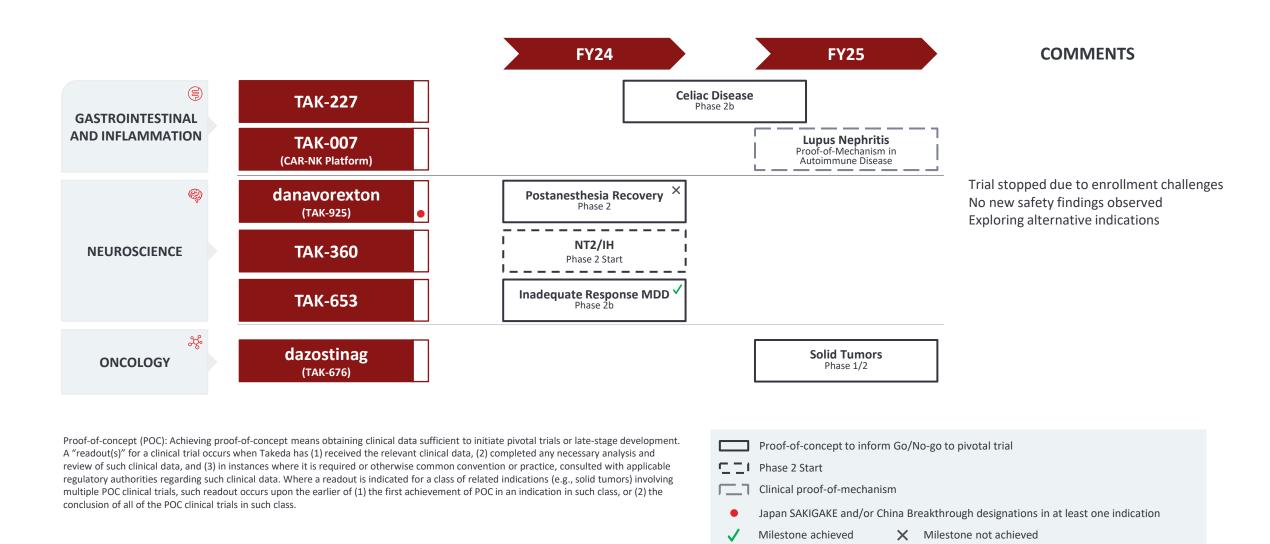




^{1.} Soticlestat DS totality of Phase 3 data suggests potential clinically meaningful benefit despite missing primary endpoint. Next step is to discuss potential filing with FDA.

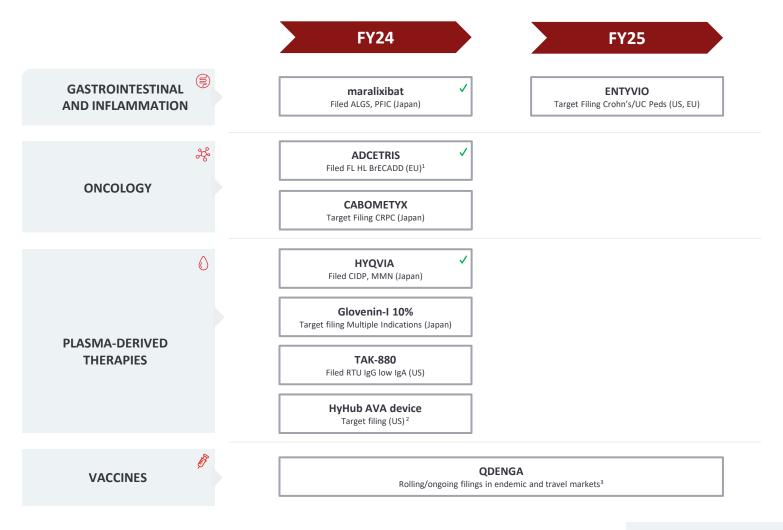
Impactful Pipeline Milestones for Early to Mid-Stage Programs Advance Science and Address Unmet Patient Needs





Important Near-Term LCM Expansions Represent Significant Growth Opportunities





- 1. Submission based on data from German Hodgkin Study Group HD21 trial
- 2. HyHub: Advanced vial access for a sterile, single-use medical device that significantly simplifies the preparation and delivery of fSCIG from vials
- 3. QDENGA approved in Vietnam (May 2024), Israel (May 2024), Switzerland (July 2024)



Glossary of Abbreviations



Regional Abbreviations:

CN: China; EU: Europe; JP: Japan; UK: United Kingdom; U.S.: United States of America

United States of America		
AATD	α1-antitrypsin deficiency	
AATD LD	α1-antitrypsin deficiency associated liver disease	
ADAMTS13	a disintegrin-like and metalloproteinase with a thrombospondin type 1 motifs 13	
ALGS	Alagille syndrome	
ALK	anaplastic lymphoma kinase	
ALL	acute lymphocytic leukemia	
AVA	Advanced Vial Access	
BID	bis in die, twice a day	
BLA	biologics license application	
BTD	breakthrough therapy designation	
CAR NK	chimeric antigen receptor natural killer cell	
СНМР	Committee for Medicinal Products for Human Use	
CIDP	chronic inflammatory demyelinating polyradiculoneuropathy	
CML	chronic myeloid leukemia	
CMV	cytomegalovirus	
CP-CML	chronic-phase chronic myeloid leukemia	
CPF	complex perianal fistulas	
CRC	colorectal cancer	
CRL	complete response letter	
CRPC	castrate-resistant prostate cancer	
сТТР	congenital thrombotic thrombocytopenic purpura	
DOAC	direct oral anti-coagulation	
DS	Dravet syndrome	

EGFR	epidermal growth factor receptor
EMA	European Medicines Agency
ESRS	European Sleep Research Society
ESS	Epworth Sleepiness Scale
FDA	U.S. Food & Drug Administration
FL	front line
fSCIG	facilitated Subcutaneous Immunoglobulin
FSI	first subject in
FY	fiscal year
GI	gastrointestinal
GvHD	graft versus host disease
H2H	head-to-head
HAE	hereditary angioedema
HemA	hemophilia A
HL	Hodgkin lymphoma
IBD	inflammatory bowel disease
IgA	immunoglobulin A
IgAN	immunoglobulin A nephropathy
IgG	immunoglobulin G
IH	idiopathic hypersomnia
IND	investigational new drug
INN	international non-proprietary name
ITP	immune thrombocytopenia
iTTP	immune thrombotic thrombocytopenic purpura
IV	intravenous
JAK	Janus kinase

LCM	lifecycle management
LGS	Lennox-Gastaut syndrome
LTE	long-term extension
mCRC	metastatic colorectal cancer
mCRPC	metastatic castrate-resistant prostate cancer
MDD	major depressive disorder
MG	myasthenia gravis
MM	multiple myeloma
MMN	multifocal motor neuropathy
MSA	multiple system atrophy
MWT	maintenance of wakefulness test
ND	newly diagnosed
NDA	new drug application
NEJM	New England Journal of Medicine
NK	natural killer
NME	new molecular entity
NMPA	(China's) National Medical Products Administration
NSCLC	non-small cell lung cancer
NT1 or 2	narcolepsy type 1 or 2
PASI	psoriasis area and severity index
PFIC	progressive familial intrahepatic cholestasis
Ph+ ALL	Philadelphia chromosome-positive acute lymphoblastic leukemia
PID	primary immunodeficiency
PK	pharmacokinetics
PMDA	Japan's Pharmaceuticals and Medical Devices Agency

POC	proof of concept
PR	platelet response
PRIME	Priority medicines scheme by EMA
PROC	platinum-resistant ovarian cancer
QD	quaque die, every day
QOL	quality of life
R/R	relapsed/refractory
RTU	ready to use
SC	subcutaneous formulation
SCT	stem cell transplant
SEM	standard error of the mean
SID	secondary immunodeficiency
SLE	systemic lupus erythematosus
soc	standard of care
TEAE	treatment emergent adverse event
TKI	tyrosine kinase inhibitor
TTP	thrombotic thrombocytopenic purpura
TYK2	tyrosine kinase 2
UC	ulcerative colitis
VEGFR	vascular endothelial growth factor receptors
vWD	von Willebrand disease
WCR	weekly cataplexy rate
ww	Worldwide