Primary Focus: Genetic Regulation

Developing a broad portfolio of transformative therapies for patients with genetic diseases

Our Mission

Our mission for Primary Focus Genetic Regulation is to discover, develop and deliver a broad portfolio of gene therapies for patients with genetic diseases. Alongside our world-renowned partners and with competitive capabilities across the value chain we are working to build a portfolio of potentially life-changing gene therapies, with a long-term goal to deliver transformational value for patients.

~7,000 diseases

Background

Often present from birth and affecting young children, nearly 7,000 human diseases are caused by mutations or deficiencies in genetic code.[1] By targeting disease at the genetic level, we can, in a single or few treatments, **significantly improve outcomes** for **serious**, **life-limiting** and **potentially fatal diseases**.

Strategic Approach

We are building a new multidisciplinary franchise for Astellas, investing in world class end-to-end capabilities across the entire gene therapy innovation process:

FOCUS

Focusing on building a portfolio of adeno-associated virus (AAV)-delivered gene therapies for the treatment of rare neuromuscular and central nervous system (CNS) diseases.

ENRICH

Developing end-to-end discovery, development, manufacturing and commercial operations to build a portfolio of candidates; starting in neuromuscular diseases with an aspiration to expand to other organs and more common diseases.

EXPAND

Partnering and collaborating with world-renowned academic groups and leading-edge biotechnology companies, to access tools and technologies to expand our portfolio of competitive projects.

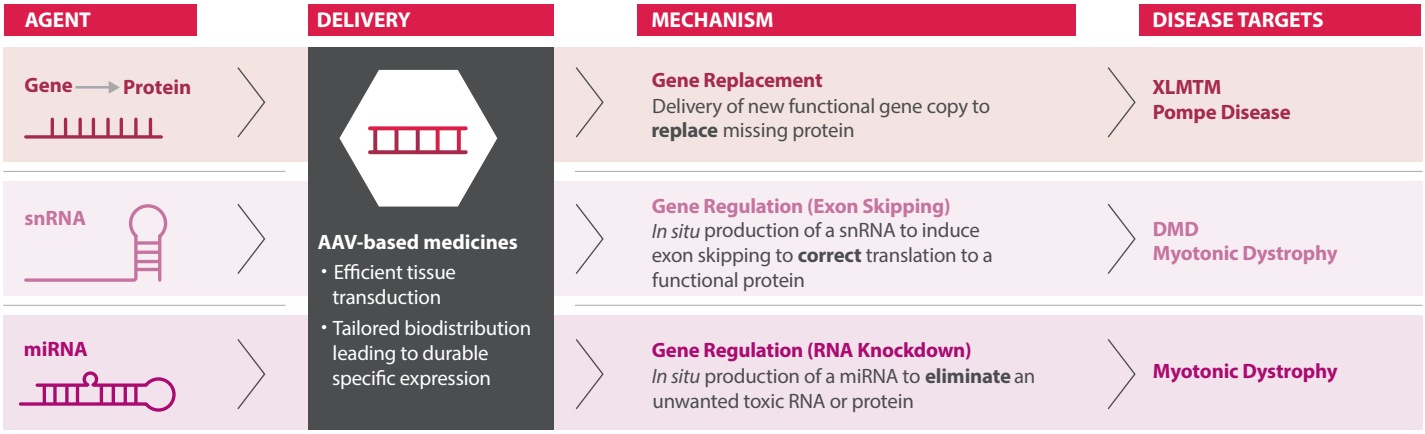
Lead program (AT132 – on clinical hold)
Candidate targeting X-linked myotubular myopathy, a serious life-threatening neuromuscular disease affecting newborn males.

Integrated facilities
Fully integrated laboratory and manufacturing facility in South San Francisco, California, U.S., and a new cutting-edge gene therapy manufacturing facility in Sanford, (North Carolina), U.S. under construction.

Effective delivery of multiple programs
Establishing a gene therapy Center of Excellence, combining a deep understanding and capability for using AAV technology, with the agility and tenacity of a biotech and Astellas' global experience and strengths.

Research Capabilities

Our innovative approach and access to novel technologies has the potential to transform outcomes for patients with rare, neuromuscular diseases, as well as more common diseases.



RNA: Ribonucleic acid, snRNA: Small nuclear RNA, miRNA: Micro RNA, AAV: Adeno-associated virus, XLMTM: X-linked myotubular myopathy, DMD: Duchenne muscular dystrophy

Pipeline – Current Status†

Current focus

Program	Mechanism	Target indication	Current phase	Origin/Partner
AT132	MTM1 gene replacement	X-linked myotubular myopathy	Phase 2 - Pivotal	astellas GENE THERAPIES
AT845	GAA gene replacement	Pompe disease	Phase 1	astellas GENE THERAPIES
AT466	Vectorized exon skipping/ vectorized RNA knockdown for DMPK	Myotonic dystrophy	Discovery	astellas GENE THERAPIES
AT808	FXN gene replacement	Friedreich's Ataxia	Discovery	astellas GENE THERAPIES
(Not disclosed)	UBE3A restoring	Angelman Syndrome	Discovery	astellas GENE THERAPIES
MDL-201	Not disclosed	Muscle disease	Preclinical	MODALIS
MDL-202	Not disclosed	Muscle disease	Preclinical	MODALIS

† Accurate as of April 2022.
MTM: Myotubularin, GAA: Acid alpha-glucosidase, DMPK: Myotonic dystrophy protein kinase, FXN: Frataxin

REFERENCES: 1. U.S. Department of Health & Human Services. Rare Diseases FAQ, Version 11/30/2017.

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