Transforming

SCIENTIFIC INNOVATION

into

HEALTHIER FUTURES

NS Pharma is a biopharmaceutical leader in delivering life-changing care to people living with complex, rare diseases

Backed by

OVER 100 YEARS

of Nippon Shinyaku Co. development expertise

NS Pharma

US subsidiary of Nippon Shinyaku Co., Ltd.

OUR **PROMISE**

At NS Pharma, we are advancing revolutionary therapies in rare disease that address unmet needs and **help people live healthier, happier lives.**

We are leaders at the forefront of transformative scientific discovery, steadfast in our long-term commitment to shepherding therapeutic advances into novel treatments for debilitating genetic disorders.

Therapeutic areas in our pipeline:

- Duchenne muscular dystrophy
- Mucopolysaccharidosis Type I and II
- Eosinophilic granulomatosis with polyangiitis (EGPA)
- GUCY2D-associated Leber congenital amaurosis (LCA1)

OUR **PROCESS**

By uniting our **strategic partnerships**, novel **scientific innovation** and expansive **organizational expertise**, we consistently drive meaningful therapeutic progress for patients facing complex, rare diseases.

NS Pharma

BOLD **INNOVATION**

Our collective expertise equips us to bring forth **unprecedented treatment options** to families living with rare diseases, raising the standard of what's possible through **dedicated partnership**.

Therapeutic mechanisms in our pipeline:

- Exon 44, 50, 51, 53 skipping
- Cell therapy
- Gene therapy
- Selective JAK1 inhibition

OUR **COMMUNITY PARTNERSHIP**

At NS Pharma, our **passionate relationships** with patients, caregivers, advocacy groups, and healthcare professionals are critical to effectively **addressing unmet needs**, understanding the integrated patient journey and seizing opportunities for **furthering impactful innovation**.

We are committed to sharing comprehensive data and encouraging our patient communities to make informed, **science-driven decisions** about their care.

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OUR PIPELINE

We partner with best-in-class organizations and medical facilities to ensure operational excellence throughout the drug development process, from clinical trials to patient delivery.

	MECHANISM	THERAPEUTIC AREA	NAME
NEUROLOGY	Exon 44 Skipping	Duchenne muscular dystrophy	NS-089/ NCNP-02 (brogidirsen)
	Exon 50 Skipping	Duchenne muscular dystrophy	NS-050/ NCNP-03
	Exon 51 Skipping	Duchenne muscular dystrophy	NS-051/ NCNP-04
	Exon 53 Skipping	Duchenne muscular dystrophy	NS-065/ NCNP-01 (viltolarsen)
	Gene Therapy	Mucopolysaccharidosis Type I	RGX-111**
	Gene Therapy	Mucopolysaccharidosis Type II	RGX-121 (clemidisogene lanparvovec)**
CARDIOLOGY/ NEUROLOGY	Cell Therapy	Duchenne muscular dystrophy cardiomyopathy	CAP-1002 (deramiocel)*
INFLAMMATORY DISEASES	Selective JAK1 Inhibition	Eosinophilic granulomatosis with polyangiitis (EGPA)	NS-229
OPHTHALMOLOGY	Gene Therapy	GUCY2D-associated Leber congenital amaurosis (LCA1)	ATSN-101***

*Nippon Shinyaku Co., Ltd. is partnering with Capricor Therapeutics, Inc., which will be responsible for the progress and development of this program.

**Nippon Shinyaku Co., Ltd. is partnering with REGENXBIO Inc., which will be responsible for the progress and development of this program.

***Nippon Shinyaku Co., Ltd. is partnering with Atsena Therapeutics, Inc., which will be responsible for the progress and development of this program

OUR PRODUCT

Our organization is strategically built to leverage vast internal industry expertise and external innovations to transform scientific breakthroughs into life-changing therapeutics.

Viltepso® (viltolarsen) injection

Indication

VILTEPSO is indicated for the treatment of Duchenne muscular dystrophy (DMD) in patients who have a confirmed mutation of the DMD gene that is amenable to exon 53 skipping. This indication is approved under accelerated approval based on an increase in dystrophin production in skeletal muscle observed in patients treated with VILTEPSO. Continued approval for this indication may be contingent upon verification and description of clinical benefit in a confirmatory trial.

Important Safety Information

In clinical studies, no patients experienced kidney toxicity during treatment with VILTEPSO. However, kidney toxicity from drugs like VILTEPSO may be possible. Your doctor may monitor the health of your kidneys before starting and during treatment with VILTEPSO.

Common side effects include upper respiratory tract infection, injection site reaction, cough, and fever.

For more information about VILTEPSO, see full Prescribing Information by following the QR code below.

Scan here to explore our pipeline and for full prescribing information

NS Pharma

OUR **PERSONAL TOUCH**

Our award-winning patient services illustrate our promise to provide dedicated partnership, driving equitable access and supporting the unique needs of every patient through their journey.

"Our role is to walk the journey with the patient, obviously helping with access and supporting them really through every step of the way... we may not have the answer, but we have the ability to get the answer."

Michelle Quinn
Patient Engagement Liaison,
NS Pharma

OUR **PURPOSE**

We are devoted to improving the **quality of life** for people living with rare diseases by **expanding novel treatment options** and **addressing the most pressing needs** of the communities we serve.

Built on humility and integrity, NS Pharma is fueled by passionate, diverse individuals who create lasting impact within the rare disease community every day.

NS Pharma

NS SUPPORT

Starting a new treatment for a rare disease can be challenging, especially for those newly diagnosed. That's why NS Support offers comprehensive support – for patients, caregivers, and families – with a personal touch.

Real people providing real support to patients and their caregivers, in coordination with their care providers.

It's more than a phone line.
It's a lifeline.

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