

March 2022

Corporate Fact Sheet

Together we soar.

JCR Pharmaceuticals Co., Ltd. is a global specialty pharmaceuticals company that is redefining expectations and expanding possibilities for people with rare and genetic diseases worldwide. Our core values - reliability, confidence, and persistence - mean that the work we do benefits all our stakeholders, including employees, partners, and patients.

We continue to build upon our 46-year legacy in Japan while expanding our global footprint with trials in the US, Europe, and Latin America. We improve patients' lives by applying our scientific expertise and unique technologies to research, develop, and deliver next-generation therapies.

Development Pipeline

We are leaders in developing therapies for rare conditions, including lysosomal storage disorders (LSDs). We are building on the development work we've done in Japan to expand our LSD pipeline:

Target Indicators	Basic Research	Animal Model Study	Process Development*	Preclinical	Clinical (Global)	Application for marketing approval	Approved/ Launch
MPS II - Hunter**	JR-141 (Jaj JR-141 (Bra						
MPS II - Hunter	JR-141 (U.S	S., EU, and Lati	n America)				
MPS I - Hurler etc.	JR-171						
Pompe disease	JR-162						
MPS IIIA - Sanfilippo A	JR-441						
MPS IIIB - Sanfilippo B	JR-446						
MPS VII - Sly	JR-443						
GM1 gangliosidosis							
Fucosidosis							
GM2 gangliosidosis - Tay-Sachs disease							
Krabbe disease							
CLN1- Batten disease infantile							
Metachromatic leukodystrophy							
CLN2- Batten disease late infantile							
Gaucher disease							
Niemann-Pick disease							
α-Mannosidosis							
Galactosialidosis							

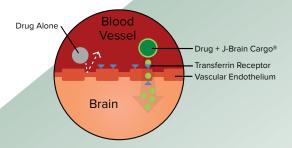
^{*}Including development of industrial scale process, testing method and formulation

For more information:

^{**}Approved in Japan March 2021 and launched in May 2021

J-Brain Cargo® Technology

Our first-in-class proprietary technology, J-Brain Cargo®, enables us to develop therapies that cross the blood-brain barrier and penetrate the central nervous system (CNS). The CNS complications of diseases are often severe, resulting in developmental delays, an impact on cognition and, above all, poor prognosis, which affect patients' independence as well as the quality of life of patients and their caregivers. With J-Brain Cargo®, we seek to address the unresolved clinical challenges of lysosomal storage disorders by delivering the enzyme to both the body and the brain.



Leadership Team



Shin Ashida Chairman, President & CEO



Toru Ashida Senior Vice President Sales and Administration Executive Director, Sales Division



Mathias Schmidt, PD, Ph.D. Vice President, Clinical Development Global Business Strategy and Business Development, ArmaGen, Inc. CEO JCR USA Inc. President & CEO



Hiroyuki Sonoda, Ph.D. Vice President Research and Corporate Strategy Executive Director, Research Division



Yoshio Hiyama, Ph.D. Senior Executive Director Production and Quality Assurance Executive Director, Production Division

Office Locations

US Headquarters:

12544 High Bluff Drive, Suite 340, San Diego, CA 92130, U.S.A.

Headquarters:

3-19 Kasuga-cho, Ashiya, Hyogo, 659-0021 Japan

Research Institute:

2-2-9 Murotani, Nishi-ku, Kobe, Hyogo, 651-2241 Japan

Bio Research Center:

1-5-4 Murotani, Nishi-ku, Kobe, Hyogo, 651-2241 Japan

JCR DO BRASIL:

Avenida das Nações Unidas, 14261 – Torre B, 27° Andar, Sala 122, Vila Gertrudes, São Paulo, Brasil

TSE 4552 | **Employees:** 785

Recent Milestones

We will begin our first global trials in the U.S., Europe, and Latin America in several disease states in 2022. Recent milestones include:

September 2020

Received Orphan Drug Designation for JR-141 (MPS II) in Japan

October 2020

First patient dosed in phase 1/2 of global clinical trial of JR-171 (MPS I)

December 2020

Filed for marketing authorization of JR-141 (MPS II) in Brazil

February 2021

Cleared FDA IND for JR-141

Received Fast Track Designation for JR-141 (MPS II) from FDA Received Orphan Drug Designation for JR-171 (MPS I) from FDA

March 2021

Obtained marketing approval of JR-141 (MPS II) from MHLW in Japan Received Orphan Drug Designation for JR-171 from European Commission

May 2021

Launch of pabinafusp alfa (MPS II) in Japan

September 2021

Received Fast Track Designation for JR-171 (MPS I) from FDA Conclusion of an exclusive co-development and commercialization agreement for JR-141 with Takeda Pharmaceutical Co., Ltd in certain regions JR-171 was granted Fast track designation

October 2021

Received PRIME Designation for JR-141 (MPS II) from EMA

January 2022

Received Orphan Drug Designation for JR-441 from European Commission

For more information:

→ jcrpharm.co.jp



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IZCARGO® received the new treatment award from WORLDSymposiumTM 2022