



# *JCR Report 2024*

JCR Pharmaceuticals Co., Ltd.

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Year Ended March 31, 2024

# Hope for the untreatable — that's JCR

We are dedicated to helping patients  
reclaim their everyday lives.  
Through innovation and a seamless approach  
from research to manufacturing,  
we create life-changing treatments  
that shape a better future.

Game-Changing  
Technologies

A Promise for a  
Better Future

A mother once said,  
“This medicine gave us hope for a life  
we never thought possible”

Giving Hope  
Where None Existed

# Creating a World Where No One Is Left Behind

For families affected by rare diseases, every day is a battle against unseen challenges. The absence of effective treatments leaves patients with difficult choices, often overshadowed by feelings of uncertainty. We are here to change that. Our mission is to listen, to act, and to transform lives.

With our cutting-edge innovations, like the groundbreaking proprietary J-Brain Cargo® technology, we've opened doors where none existed before. This world-first breakthrough in crossing the blood-brain barrier isn't just a scientific achievement—it's a promise. A promise to give patients and their families more options, more hope, and a chance at a better future.

But innovation alone isn't enough. That's why we've established a global network spanning Europe, Japan, USA, and South America. Our international teams work tirelessly to navigate diverse regulations and accelerate the delivery of life-changing therapies. Together, we're breaking barriers—not just in science but across borders—to bring treatments to those who need them most.

We believe in the potential within every patient. We believe in pushing boundaries, embracing challenges, and moving forward. Above all, we believe in a future where possibilities are endless, and no one is left behind.

# Research & Innovation

## Breaking Barriers, Bringing Hope

For over 350 million people worldwide living with rare diseases, the lack of treatment options is not just a statistic—it's their daily reality. Behind the numbers are individuals and families searching for hope, navigating lives shaped by limitations and uncertainty.

Rare diseases, defined by their low prevalence, include as many as 5,000 to 8,000 distinct conditions. While each affects only a small number of patients, collectively, they represent a significant and urgent challenge. These diseases, often genetic or progressive, impose profound physical, emotional, and social burdens. Yet, 95% of them have no approved treatment, leaving countless lives impacted by barriers that seem insurmountable.

At JCR, we see these challenges not as roadblocks but as our mission. Tackling what others might deem impossible is at the heart of what we do.

## The Innovation of J-Brain Cargo®: Hope Delivered

Among the most formidable challenges in medicine are brain-related rare diseases. The blood-brain barrier, which protects the brain from harmful substances, also prevents most drugs from reaching the brain. This natural defense mechanism has historically left patients with neurological symptoms without effective treatments.

JCR's J-Brain Cargo® technology changes this narrative. By enabling therapies to cross the blood-brain barrier, it offers a breakthrough that was once thought impossible. This pioneering approach has opened new possibilities for patients and families facing conditions previously deemed untreatable.

A shining example is JR-141, a treatment for Hunter syndrome (Mucopolysaccharidosis Type II). Hunter syndrome, a lysosomal storage disorder, stems from enzyme deficiencies that cause harmful substances to accumulate in cells. While traditional therapies struggle to address neurological symptoms, JR-141, powered by J-Brain Cargo®, offers hope for both systemic and central nervous system symptoms. Approved and available in Japan, JR-141 is now in clinical trials across the US, Europe, and South America, paving the way for patients worldwide to access transformative care.



How many people are affected by rare diseases?

**350-400 million**  
people worldwide



What causes rare diseases?

**80% of**  
rare diseases  
are genetic



How many rare diseases are there?

**There are between**  
**5,000-8,000**  
rare diseases

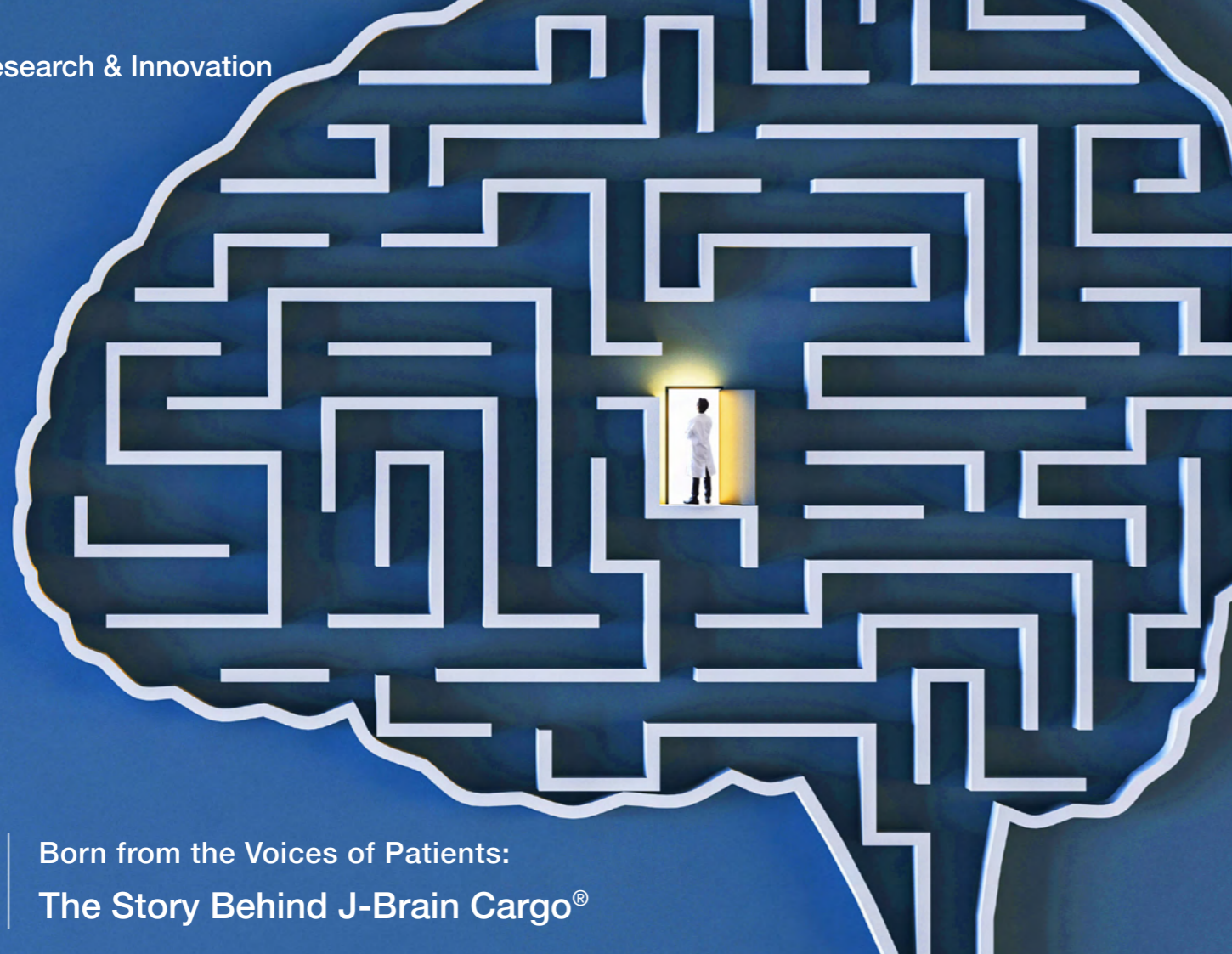
\*Source: IFPMA. Rare diseases: shaping a future with no-one left behind. 2017.

Building on this success, JCR is expanding the application of J-Brain Cargo® to other lysosomal storage disorders, with multiple clinical trials underway globally. For those living with ultra-rare conditions like Fucosidosis—affecting fewer than 120 people worldwide—we are determined to bring hope where it's needed most.

At JCR, innovation is more than technology; it's about transforming possibilities into realities and delivering hope to those who need it most.

## Shaping a Future of Possibilities

Addressing rare diseases is a relentless pursuit, with no easy solutions in sight. Yet, we remain steadfast in our commitment to creating new treatment options. Every breakthrough we achieve is a step closer to a future where hope reaches every patient. With passion and innovation, we will continue to push boundaries, overcome challenges, and turn possibilities into realities. Our journey has only just begun.



Born from the Voices of Patients:  
The Story Behind J-Brain Cargo®

## “Please, save my child.” Those words changed my life as a researcher.



**Hiroyuki Sonoda**  
Director, Senior Managing Executive Officer,  
Research, Executive Director,  
Research Division

When I joined JCR in 2003, I began working on lysosomal storage disorders—a group of rare diseases caused by enzyme deficiencies that lead to harmful substances accumulating in cells. These diseases often result in severe, systemic symptoms. However, at that time, it was considered impossible to deliver medicines to the brain to address central nervous system symptoms.

In 2005, I attended a patient advocacy meeting for mucopolysaccharidosis (MPS). I saw children in wheelchairs, others lying in hospital beds with feeding tubes, and families sharing their heartbreaking experiences through tears. A woman sitting next to me mistook me for a doctor and pleaded, “Please save my son.” I explained that I was a researcher at a pharmaceutical company, not a physician. Her response was simple yet profound: “That’s fine—just listen to me.”

Her son, like many with this type of MPS, began showing symptoms around age three. As the disease progressed, he lost the ability to communicate. At that

moment, I felt utterly powerless. I wasn’t a doctor. I couldn’t provide immediate help. But her words left a mark on my heart:

“As a researcher, there must be something I can do.”

### Confronting the “Impossible”

At the time, delivering drugs to the brain was deemed impossible due to the blood-brain barrier—a natural defense mechanism that blocks external substances, including medicines, from reaching the brain. Yet, treating MPS and its neurological symptoms required overcoming this barrier.

“No matter what, I will find a way to cross this barrier.”

This resolution fueled years of relentless research, marked by failure after failure. Test results often showed zero progress, and hope seemed elusive. But I couldn’t forget the mother’s plea: “Please save my son.”

Eventually, I identified a potential solution: leveraging the transferrin receptor (TfR), which the brain uses to absorb iron. By applying this mechanism, we hypothesized that drugs could cross the blood-brain barrier. Initial experiments in mice confirmed that the medicine reached the brain, and subsequent trials in monkeys yielded similar results. Finally, in 2017, clinical trial data provided undeniable evidence:

“The drug has crossed the blood-brain barrier and reached the brain.”

At that moment, all the years of struggle felt worthwhile. We had achieved what was once thought impossible.

### J-Brain Cargo®: Are Go!

This revolutionary technology, J-Brain Cargo®, broke through the once-impenetrable blood-brain barrier, enabling medicines to reach the brain and transform treatment possibilities for rare diseases. Its first application, JR-141, a therapy for MPS Type II (Hunter syndrome), has been approved in Japan and is now undergoing clinical trials in the United States, Europe, and South America.

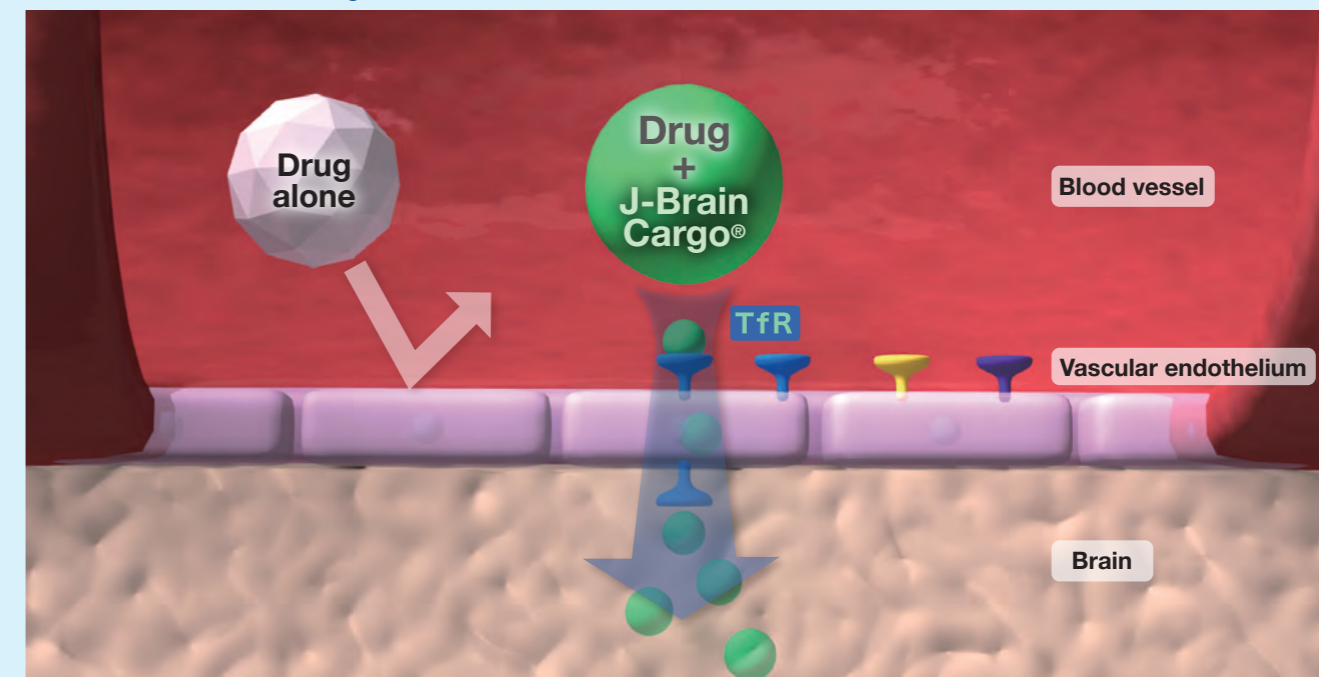
Without the heartfelt pleas of children and their families, and the words of one mother in particular, J-Brain Cargo® might never have come to life. Today, it stands as a beacon of hope, offering new possibilities to those who had none.

### “Patient Voice is Our Greatest Motivation”

The voices of patients and their families were the driving force behind this research. “Please save us.” Responding to that call, we took on challenges others deemed impossible and pushed past barriers.

At JCR, we will continue refining our technologies and striving to deliver hope to every patient still waiting for a breakthrough.

### Mechanism for J-Brain Cargo®



# J-Brain Cargo®: Breaking Barriers, Pioneering the Future

Born from an unwavering determination to “save lives,” the J-Brain Cargo® technology has achieved what was once considered impossible. It laid the foundation for a groundbreaking advancement: the world’s first medicine capable of addressing the unmet needs of rare diseases affecting the brain.

## The Journey of JR-141: From Innovation to Impact

Overcoming the barriers of the blood-brain barrier, JR-141—a therapy for MPS Type II developed using J-Brain Cargo®—represents a revolutionary breakthrough. Designed to address the neurological symptoms that were previously untreatable, JR-141 has redefined what is possible for patients with rare diseases.

Rare disease clinical trials present unique challenges, from the small number of eligible patients to the complexities of treating children. Despite these hurdles, in 2017, clinical trials for JR-141 began. Within just six months, the target of enrolling 12 patients was achieved,

thanks to the collective efforts of families and healthcare providers united by a shared urgency: to bring treatment to patients as quickly as possible. The trials progressed smoothly, with data demonstrating significant efficacy.

In 2018, JR-141 received a designation under Japan’s Sakigake Fast-Track Review system, accelerating its path to approval. By March 2021, just four years after the trials began, JR-141 was officially approved in Japan under the brand name Izcargo®. This achievement was a testament to the relentless dedication of JCR’s researchers and their commitment to delivering treatments to patients without delay.

## Global Recognition for J-Brain Cargo®

JR-141, powered by J-Brain Cargo®, has earned international acclaim for its groundbreaking contributions. In 2022, it received the New Treatment Award at the 18th Annual WORLDSymposium™ for its innovation in the field of lysosomal storage disorders. This honor highlighted JCR’s success in overcoming challenges long deemed insurmountable and bringing an effective treatment to market.

“This technology opens the door to new treatments.”  
“Delivering medicine to the brain—could this be applied to other diseases as well?”

These were just some of the questions and accolades voiced by global experts during the award ceremony, reflecting the transformative potential of JCR’s innovation.

## Expanding Horizons: Beyond Barriers

Building on the success of JR-141, JCR is advancing over 17 pipeline products within the lysosomal storage disorder space. But the journey doesn’t stop there. The J-Brain Cargo® technology is now being explored for applications in other intractable diseases and even in gene therapy.

We are driven by the vision of delivering treatments that were once unimaginable, bringing them to more patients, faster. Our journey is one of constant discovery, breaking through barriers and stepping into a future filled with new possibilities.



IZCARGO® I.V. infusion, recombinant treatment for mucopolysaccharidosis II



## Development Pipeline of lysosomal storage disorder therapeutics

Basic Research	Preclinical	Clinical (Phase I/II)	Clinical (Phase III)	Review Period	Approved
<b>JR-194</b> (Batten disease type I)	<b>JR-471</b> Fucosidosis	<b>JR-171</b> MPS I (Hurler syndrome, etc.)	<b>JR-141</b> MPS II (Hunter syndrome)	<b>JR-141</b> MPS II (Hunter syndrome)	<b>JR-141</b> MPS II (Hunter syndrome)
<b>Krabbe disease</b>	<b>JR-162</b> Pompe disease	<b>JR-441</b> MPS IIIA (Sanfilippo A syndrome)			
<b>GM1 gangliosidosis</b>	<b>JR-443</b> MPS VII (Sly syndrome)	<b>JR-446</b> MPS IIIB (Sanfilippo B syndrome)			
<b>CLN2</b> (Batten disease type II)	<b>JR-479</b> GM2 gangliosidosis (Tay-Sachs, Sandhoff disease)				
<b>Gaucher disease</b>					
<b>α-Mannosidosis</b>					
<b>Niemann-Pick</b>					
<b>Metachromatic leukodystrophy</b>					
<b>Galactosialidosis</b>					

As of January 2025

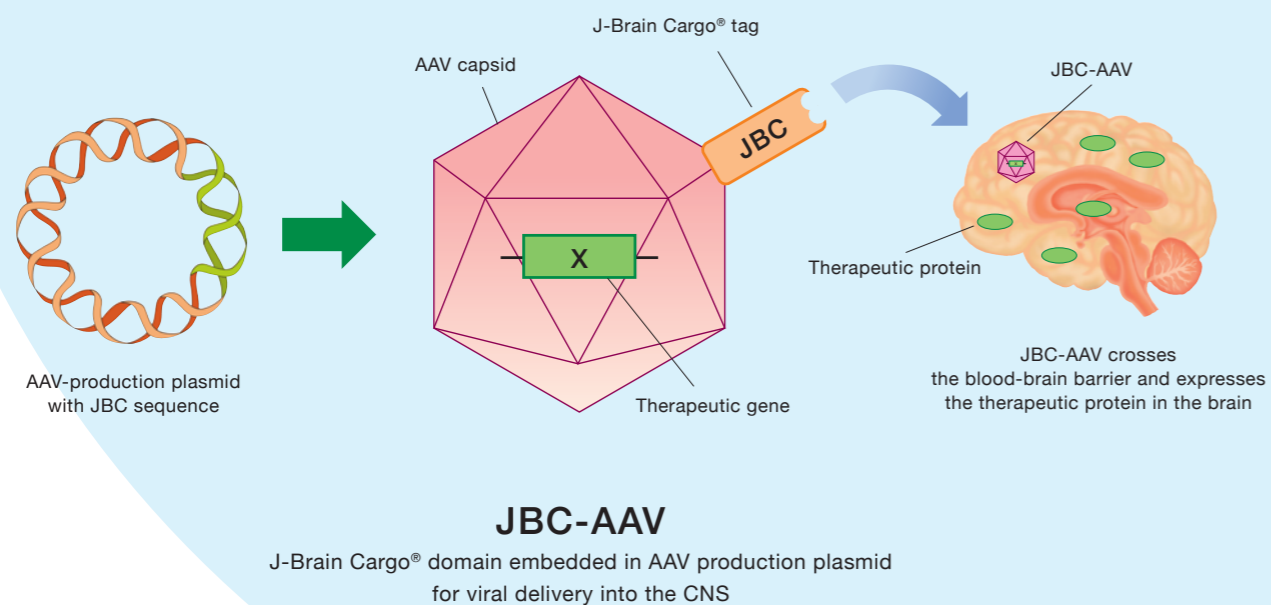
# J-Brain Cargo®: Unlocking the Future of Gene Therapy

Many rare diseases are caused by genetic mutations or deficiencies, yet delivering treatments to the brain has long been a significant challenge. The blood-brain barrier, while vital for protecting the brain, has also limited therapeutic options for patients and their families.

To overcome this barrier, JCR has adapted its groundbreaking J-Brain Cargo® technology, opening new doors in the field of gene therapy. One promising application is its integration with adeno-associated virus (AAV), a widely recognized gene delivery vector. While AAV has shown potential, its effectiveness in reaching the brain and muscles has been limited, and concerns over liver toxicity remain a significant challenge.

Since 2017, we have been pioneering the fusion of J-Brain Cargo® with AAV technology. This innovation enables the efficient delivery of genes to brain cells while minimizing liver toxicity. In preclinical trials with mice and monkeys, the technology demonstrated the ability to cross the blood-brain barrier effectively, delivering genes precisely to the brain and significantly reducing associated risks.

## Applying J-Brain Cargo® to Gene Therapy



## A Breakthrough Garnering Global Attention

This groundbreaking achievement was unveiled in 2024 at the 7th International Lysosomal Disease Forum and the 31st Annual Congress of the European Society of Gene and Cell Therapy (ESGCT). Demonstrating the ability to "pass through the blood-brain barrier" in AAV-based gene therapy captured the attention of researchers and healthcare professionals worldwide, sparking significant excitement.

"This could be a new therapeutic option."  
"Could this technology also be applied to other intractable diseases?"

The presentations generated a wave of questions, highlighting the excitement and potential surrounding this innovative approach.

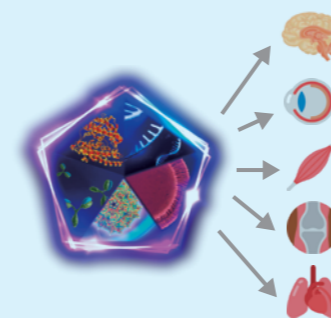
## Beyond Gene Therapy

JCR is extending the applications of J-Brain Cargo® beyond gene therapy, exploring its use in antibody-based and nucleic acid therapies. Collaboration with global partners is also a key part of this journey. In 2023, we began a research collaboration with Alexion, focusing on neurodegenerative diseases, and reached its first milestone. Additionally, a partnership with Angelini Pharma was established to develop innovative treatments for epilepsy.

We are driven by a singular goal: to create treatments that were once deemed impossible, reaching more patients in less time. Through innovative partnerships and relentless research, we are not just advancing medicine—we are reimagining what's possible for patients around the world.

## Shaping the Future with Our Own Technology

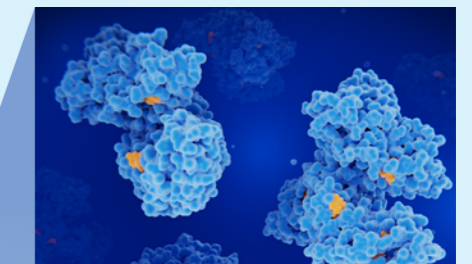
Widely applicable technology based on J-Brain Cargo®



- Expansion of partnering opportunities
- Increasing R&D collaborations



## Creation of breakthrough therapeutics in various disease areas



- Lysosomal Storage Diseases
- Neurodegenerative Diseases
- Ocular Diseases
- Orthopedic Diseases
- Muscular Diseases

# Expanding Globally: Delivering Innovation to Patients Worldwide

JCR is committed to transforming the lives of patients with rare diseases by ensuring they have access to innovative treatments, no matter where they live. With an integrated global framework, our teams across Europe, Japan, the United States, and South America are working together to accelerate clinical trials and regulatory processes.

Central to this effort is our pioneering work in lysosomal storage disorders. By harnessing the power of J-Brain Cargo® technology, we are overcoming challenges that once rendered brain-targeted therapies unattainable.

JR-141, our treatment for MPS Type II, received approval in Japan in 2021, marking the start of a journey to reach patients around the world. Now in clinical trials across the United States, Europe, and South America, it represents a step toward broader access to life-changing care. Building on this progress, we are advancing therapies like JR-171 for MPS Type I and JR-441 for MPS IIIA, opening new doors of hope for patients and their families.

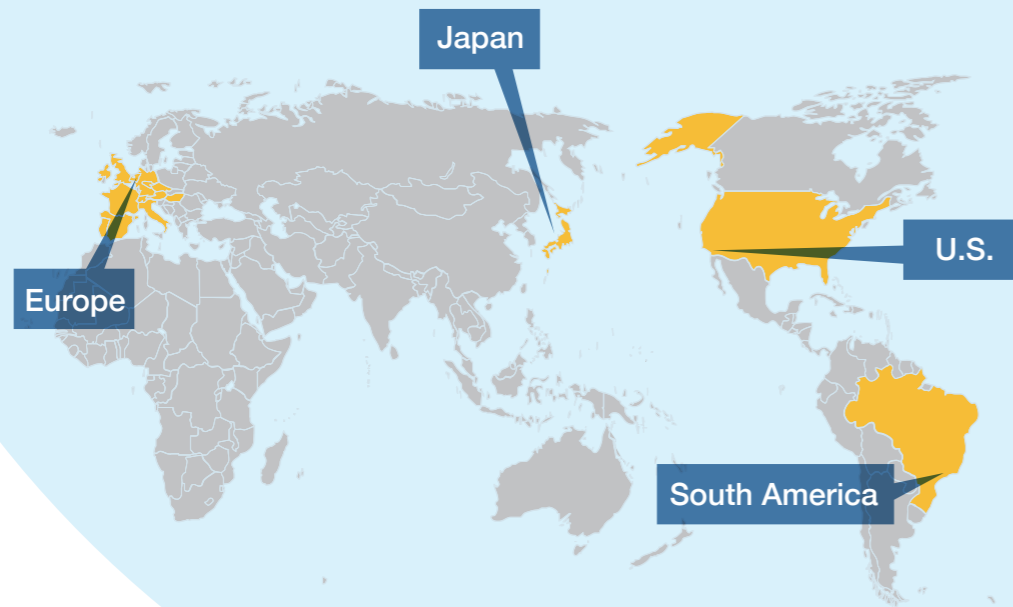
## More Than Market Expansion

Our global initiatives are not simply about expanding markets. They are about addressing the unique needs and regulatory requirements of each region to ensure that every patient receives the care they need.

Through our collaborative approach, “Team JCR” integrates global expertise to streamline development and deliver treatments with precision and speed. By partnering with leaders in neurodegenerative diseases and epilepsy treatment, we are unlocking new possibilities for delivering therapies to previously unreachable areas of the brain. These efforts exemplify the power of innovation and partnership in addressing the unmet needs of patients worldwide.

What drives us at JCR is the belief that every patient deserves a chance at a better life. With innovation as our compass and collaboration as our strength, we are shaping a future where treatments once thought impossible become a reality for those who need them most.

### Global Development Sites



Development Pipeline and Progress (As of January 2025)

■ Lysosomal Storage Disorders ■ Other biopharmaceuticals

Code	Nonproprietary Name	Indication	Region	Preclinical	Clinical trials	Approved
JR-141	BBB-penetrating iduronate-2-sulfatase (rDNA origin)	Mucopolysaccharidosis II (Hunter syndrome)	Global	Phase III		
JR-142	Long-acting growth hormone (rDNA origin)	Pediatric growth hormone deficiency	Japan	Phase III		
JR-171	BBB-penetrating α-L-iduronidase (rDNA origin)	Mucopolysaccharidosis I (Hurler syndrome, etc.)	Global	Phase I/II		
JR-441	BBB-penetrating heparan N-sulfatase (rDNA origin)	Mucopolysaccharidosis III-A (Sanfilippo syndrome type A)	Global	Phase I/II		
JR-446	BBB-penetrating α-N-acetylglucosaminidase (rDNA origin)	Mucopolysaccharidosis III-B (Sanfilippo syndrome type B)	Japan	Phase I/II		
JR-471	BBB-penetrating α-L-fucosidase (rDNA origin)	Fucosidosis	—	Preclinical		



# Our Mission for a Sustainable Tomorrow

We are dedicated to delivering new treatment opportunities to patients and families facing rare and ultra-rare diseases worldwide. Through our expertise in R&D and manufacturing, we embrace the principle of No one will be left behind – a cornerstone of the UN Sustainable Development Goals (SDGs).

We strive to provide high-quality medicines reliably while advancing environmentally responsible production. By setting clear goals and taking action at every level, we are working toward a more sustainable and inclusive future.

Listening to patients and families drives us to achieve sustainability that only JCR can offer. We remain committed to earning the trust of all who depend on us.



**Toru Ashida**  
Director, Senior Managing Executive Officer for Sustainability

## Our Commitment to Sustainability

Since our founding in 1975, JCR has been guided by the philosophy of "Contributing to people's health through pharmaceuticals". With advanced biotechnology and regenerative medicine, we aim to develop groundbreaking treatments for unmet medical needs, particularly in rare and ultra-rare diseases.

As global environmental and social challenges evolve, JCR focuses on creating long-term value and contributing to a sustainable society. Our key areas are Rare Diseases, Environment, Society, and Corporate Governance.

Among these, Rare Diseases are at the heart of what we do. By developing treatments, improving access, and building new business models for ultra-rare diseases, we tackle challenges that only JCR can address. We strive to build a society where no one is left behind.

## Core Sustainability Initiatives

Rare Diseases (RD)		
<ul style="list-style-type: none"> <li>• Providing treatment options for rare diseases</li> </ul>		
Environment (E)	Society (S)	Corporate Governance (G)
<ul style="list-style-type: none"> <li>• Environmental Responsibility</li> </ul>	<ul style="list-style-type: none"> <li>• Innovative Technologies</li> <li>• Global Biopharmaceutical Supply</li> <li>• Talent Development</li> </ul>	<ul style="list-style-type: none"> <li>• Ethical Governance</li> </ul>

## Sustainability Governance

In July 2022, JCR established the Sustainability Advisory Committee, Sustainability Committee, and Environmental Committee. Working closely with management, these committees drive strategies and initiatives to achieve sustainability that only JCR can deliver.

### Sustainability Advisory Committee

Comprised of directors and officers, the committee advises on matters submitted to the Board by the Sustainability Committee.

### Sustainability Committee

Chaired by the Sustainability Officer and composed of representatives from each division, the committee identifies material issues, evaluates ESG initiatives, monitors progress, and reports to the Board of Directors.

### Environmental Committee

Comprised of internal directors and selected employees, the committee addresses environmental risks from business activities and promotes environmentally conscious practices.

Material Issues

Category	Key issues	Initiatives	Key Indicator	2023 Result	
				Outcomes	Activities
<b>RD</b> Rare Diseases	Providing treatment options for rare diseases	<ul style="list-style-type: none"> <li>Development of innovative treatments for rare and ultra-rare diseases</li> <li>Patient-centered product initiatives and activities</li> <li>Expanding treatment opportunities</li> <li>Building sustainable business models for rare diseases</li> </ul>	<ul style="list-style-type: none"> <li>Number of clinical trials initiated (Target: 5 by FY2027)</li> </ul>	<ul style="list-style-type: none"> <li>1 trial</li> </ul>	<ul style="list-style-type: none"> <li>Global Phase I/II trial initiated for JR-441 enzyme replacement therapy for MPS IIIA</li> <li>Engaged with patient and family groups in 16 countries (36 organizations) to understand needs.</li> <li>Conducted employee-led awareness events under the RARE DISEASE Project</li> <li>Expanded treatment options with Izcargo® for intravenous infusion</li> </ul>

Category	Key issues	Initiatives	Key Indicator	2023 Result	
				Outcomes	Activities
<b>E</b> Environment	Environmental Care	<ul style="list-style-type: none"> <li>Low-carbon operations</li> <li>Reduced environmental impact</li> </ul>	<ul style="list-style-type: none"> <li>CO<sub>2</sub> emission reduction rate via recycling industrial waste</li> </ul>	<ul style="list-style-type: none"> <li>47%</li> </ul>	<ul style="list-style-type: none"> <li>Rental uniforms made from recycled materials</li> <li>Recycled used uniforms</li> <li>Promoted paperless operations</li> <li>100% recycled toilet paper</li> <li>Shifted company cars to electric</li> <li>Ended bottled drinks at executive meetings</li> </ul>

Category	Key issues	Initiatives	Key Indicator	2023 Result	
				Outcomes	Activities
<b>S</b> Society	Innovative Foundational Technologies	<ul style="list-style-type: none"> <li>Creating innovative technologies</li> <li>Forming strategic partnerships</li> </ul>	<ul style="list-style-type: none"> <li>Partnerships</li> </ul>	<ul style="list-style-type: none"> <li>2</li> </ul>	<ul style="list-style-type: none"> <li>Collaboration with Alexion for nucleic acid drugs using J-Brain Cargo® technology</li> <li>Global partnership with Angelini Pharma for epilepsy treatment development</li> </ul>

Category	Key issues	Initiatives	Key Indicator	2023 Result	
				Outcomes	Activities
<b>S</b> Society	Global Supply System for Biopharmaceuticals	<ul style="list-style-type: none"> <li>Advanced biopharmaceutical manufacturing technologies</li> <li>Enhanced quality and supply systems</li> </ul>	<ul style="list-style-type: none"> <li>Regulatory findings by authorities (Target: 0)</li> <li>Quality-related shortages or recalls (Target: 0)</li> </ul>	<ul style="list-style-type: none"> <li>0</li> <li>0</li> </ul>	<ul style="list-style-type: none"> <li>Expanded Growject® production for stable supply</li> <li>Streamlined processes</li> </ul>

Category	Key issues	Initiatives	Key Indicator	2023 Result	
				Outcomes	Activities
<b>S</b> Society	Talent Development	<ul style="list-style-type: none"> <li>Building a dynamic talent portfolio</li> <li>Promoting Diversity, Equity &amp; Inclusion</li> <li>Advancing Workstyle Reform</li> <li>Promoting Skill Transfer</li> </ul>	<ul style="list-style-type: none"> <li>Average female hiring ratio for new graduates (past 5 years)</li> <li>Training cost per employee</li> </ul>	<ul style="list-style-type: none"> <li>44.7%</li> <li>95,000 yen</li> </ul>	<ul style="list-style-type: none"> <li>JCR Academy for global leader development (Phase 1 complete, Phase 2 started)</li> <li>On-site daycare and provided childcare subsidies</li> <li>"Kurumin" and "Eruboshi (Level 2)" certified</li> <li>"Hyogo-Kobe Women's Empowerment (Mimosa) Company" certified</li> </ul>

Category	Key issues	Initiatives	Key Indicator	2023 Result	
				Outcomes	Activities
<b>G</b> Corporate Governance	Ethical Governance	<ul style="list-style-type: none"> <li>Ethical Governance</li> <li>Ensuring Transparency</li> <li>Fostering an open, accountable culture</li> <li>Advancing Risk Management</li> </ul>	<ul style="list-style-type: none"> <li>Executive-employee communication frequency</li> <li>Board evaluation implementation and action rate (Target: 100%)</li> <li>Independent director ratio</li> <li>Whistleblowing cases addressed</li> </ul>	<ul style="list-style-type: none"> <li>26</li> <li>100%</li> <li>58.3%</li> <li>16</li> </ul>	<ul style="list-style-type: none"> <li><b>[ Communication Activities ]</b> <ul style="list-style-type: none"> <li>Run &amp; Walk Event</li> <li>Executive-employee lunch meetings</li> <li>Improvement Proposal Awards Ceremony</li> <li>Sales Division General Meeting</li> <li>JCR Group Workshops and Visits</li> <li>Employee Financial Results Briefing</li> </ul> </li> <li><b>[ Risk Management Activities ]</b> <ul style="list-style-type: none"> <li>Risk Management Promotion Meetings twice annually (July and January)</li> </ul> </li> <li><b>[ Compliance Training ]</b> <ul style="list-style-type: none"> <li>Compliance training sessions four times annually</li> </ul> </li> </ul>

\*See Corporate Governance Report for details.

Our Contribution to Rare Diseases

RARE DISEASE Project

The RARE DISEASE Project is a cross-functional internal initiative built around the motto: "What JCR can do for rare diseases." Its goals include raising awareness, sharing information within the company, and collaborating with patient associations and support organizations. To encourage broad participation, the project operates on a two-year rotating term without fixed members. Key initiatives include:

- Awareness activities such as wearing official badges and fundraising for Rare Disease Day (RDD), organizing global campaigns for MPS Awareness Day, and hosting public lectures.
- Sharing event reports, such as participation in patient support group activities, and organizing internal lectures.



■ Highlights from FY2023

- In 2023, we had the privilege of inviting patients and their families to share their stories at a company event. For those unable to attend, a recording was made available, ensuring the message of awareness reached every corner of our organization.
- Members of the RARE DISEASE project also joined several gatherings hosted by patient and family associations throughout the year, strengthening connections and deepening understanding.
- December 2023 saw us welcome students from Kobe Kaisei Girls' High School, Osaka Meisei High School, and National Institute of Technology, Tsuruoka College for the RDD High School Study Tour (JCR Internship). Through engaging presentations and open discussions led by our team, the students explored the world of rare diseases and gained insight into our mission.
- To commemorate Rare Disease Day in February

2024, we organized a series of initiatives, including charity fundraisers, creative activities like origami crane posters, and an awareness-focused crossword puzzle, all aimed at fostering understanding and compassion.

MPS Awareness Day (Global Mucopolysaccharidosis Awareness Day)

In FY2021, JCR launched global awareness initiatives to support MPS Awareness Day, held on May 15 and organized by the U.S.-based MPS Society. In FY2023, JCR continued these efforts by inviting employees across all sites to submit photos featuring purple—the awareness color—and messages of support for MPS awareness. These photos were shared on our internal bulletin board and made available as downloadable posters. Donations were made to the MPS Society based on the number of submissions received. Additional activities included wearing handmade purple ribbon badges and sharing MPS-related newsletters internally.

Rare Disease Day (RDD)

JCR has supported Rare Disease Day (RDD) since FY2015. People living with rare and intractable diseases face unique challenges, as the small number of patients and the complexity of these conditions often hinder advancements in diagnostics and treatment development. RDD began in Sweden in 2008 to improve the quality of life for rare disease patients through better diagnosis and treatment. The initiative aims to bridge patients and society, raising awareness and fostering greater understanding of rare and intractable diseases worldwide.



For more details on our sustainability initiatives, please visit our website. <https://www.jcrpharm.co.jp/en/site/en/sustainability/index.html>

**Our Core Principles**

At JCR, we are dedicated to delivering high-quality, innovative pharmaceuticals and medical devices to society. To achieve this, we prioritize management practices that are lawful, transparent, and objective. We also work to enhance corporate value while protecting shareholder interests by establishing and maintaining an effective internal control system. Regular evaluations of this system ensure we fulfill our corporate social responsibilities.

Compliance is central to our operations. We strictly adhere to laws, global standards, and industry regulations, while cultivating a culture of integrity and strong ethics in all our daily business activities.

**Overview of Governance Structure**

JCR operates as a company with a Board of Corporate Auditors, comprising a Board of Directors with 11 members (including 6 independent directors) and a Board of Corporate

Auditors with 5 members (all independent auditors). An accounting auditor is also appointed.

In addition to these bodies, JCR has established several committees and councils, including the Executive Management Committee, Nomination and Compensation Advisory Committee, Sustainability Advisory Committee, Management Meeting, Internal Audit Department, Internal Control Committee, Compliance Committee, Sustainability Committee, Safety and Health Committee, Environmental Committee, Donation Review Committee, and Risk Management Promotion Council.

This governance structure is appropriately scaled and aligned with JCR's current business operations, ensuring efficient management. Furthermore, the inclusion of 6 independent directors and 5 independent auditors strengthens transparency, objectivity, and the independence of management oversight.

**Skill Matrix of Directors and Audit & Supervisory Board Members** (As of October 31, 2024)

	Name	Position	Skill														
			Overall Management	Industry Knowledge	Global Experience	R&D	Production	Sales	ICT	Administrative Experience	Legal Affairs	Tax, Finance and Accounting	Sustainability	Risk Management	Other		
Board of Directors	Shin Ashida	Chairman, President, CEO	●														
	Toru Ashida	Senior Managing Executive Officer	●	●				●					●				
	Hirofumi Sonoda	Senior Managing Executive Officer		●		●								●			
	Yoshio Hiyama	Managing Executive Officer			●		●										
	Andrea Spezzi	Managing Executive Officer	●	●	●	●							●				
	Takashi Suetsuna	Outside Director (Independent)			●						●	●				●	
	Toshihide Yoda	Outside Director	●	●		●											
	Yuko Hayashi	Outside Director (Independent)	●							●				●			Diversity and Inclusion
	Yutaka Atomi	Outside Director (Independent)		●		●										●	
	Philippe Fauchet	Outside Director (Independent)	●	●	●												Business Development, Medical Affairs, Public Relations and Government Affairs
Marc Dunoyer	Outside Director		●	●	●												
Audit & Supervisory Board	Kazumasa Oizumi	Outside Audit (Independent)	●														Audit Practice
	Kazuhiko Yamada	Outside Audit (Independent)	●								●		●		●		
	Kenjiro Miyatake	Outside Audit (Independent)	●	●													
	Takeshi Komura	Outside Audit (Independent)	●										●				
	Shuichi Tani	Outside Audit (Independent)	●	●									●				

See the "Corporate Governance Report" for details <https://www.jcrpharm.co.jp/en/site/en/ir/governance.html>

As of September 30, 2024

**Company Profile**

Corporate Name	JCR Pharmaceuticals Co., Ltd.
Headquarters	3-19 Kasuga-cho Ashiya, Hyogo, 659-0021 Japan
Representative	Shin Ashida, Chairman, President, CEO and COO
Founded	September 1975
Paid-In Capital	9,061 million yen
Employees	997 (Consolidated) 951 (Non-Consolidated)

**Consolidated Subsidiaries**

- Chromatech Co., Ltd.
- JCR Engineering Co., Ltd.
- JCR USA, Inc. (U.S.)
- ArmaGen, Inc. (U.S.)
- JCR DO BRASIL FARMACÊUTICOS IMPORTAÇÃO E EXPORTAÇÃO LTDA. (Brazil)
- JCR Europe B.V. (The Netherlands)
- JCR Luxembourg S.A. (Luxembourg)
- JCR INTERNATIONAL SA (Switzerland)

**Equity Method Affiliates**

- AlliedCel Corporation (Joint venture)

**Stock Information**

Listed on	Tokyo Stock Exchange Prime Market
Securities Code	4552
Total Number of Outstanding Shares	129,686,308
Transfer Agent for Common Stock	Sumitomo Mitsui Trust Bank, Limited 1-4-1, Marunouchi, Chiyoda-ku, Tokyo
Accounting Auditor	Deloitte Touche Tohmatsu LLC
Number of Shareholders	22,888

**Principal Shareholders**

	(Unit: 1,000)
Name of shareholder	Number of shares held
MEDIPAL HOLDINGS CORPORATION	29,131
The Master Trust Bank of Japan, Ltd. (Trust account)	10,976
Future Brain Co., Ltd.	8,711
Custody Bank of Japan, Ltd. (Trust Account)	7,820
The Nomura Trust and Banking Co., Ltd. (Trust account: A)	6,298
Kissei Pharmaceutical Co., Ltd.	4,918
Sumitomo Pharma Co., Ltd.	3,400
Mochida Pharmaceutical Co., Ltd.	2,200
Employee Shareholding Association of JCR Pharmaceuticals Co., Ltd.	1,285
National University Corporation Kyoto University	1,000

\* The Company holds 3,922,502 shares of treasury stock, which are not included in the above table.

**Editorial Policy**

The JCR Report 2024 integrates financial and non-financial information, including sustainability initiatives, to highlight our progress toward realizing our management vision and showcasing what only JCR can achieve. The report is crafted to provide a clear understanding to a wide range of stakeholders.

To emphasize JCR's strengths, the content has been carefully refined and streamlined.

See our website for business details

<https://www.jcrpharm.co.jp/en/site/en/index.html>

**Reporting Period**

FY2023 (April 1, 2023 – March 31, 2024)

\*Includes some content from FY2024.

**Reporting Scope**

JCR Group (JCR Pharmaceuticals Co., Ltd., consolidated subsidiaries, and equity-method affiliates)

\*Any deviations from this scope are noted accordingly.

**Units of Measurement**

Figures are generally rounded down to the nearest unit.

Amounts displayed in billions of yen are rounded to the nearest whole number.

**Caution Regarding Forward-Looking Statements**

The JCR Report 2024 includes forward-looking statements based on the information available to the company. These statements involve known and unknown risks and uncertainties. Actual outcomes may differ significantly due to factors such as economic downturns, regulatory changes, product launch delays, competitive pressures, weakened sales performance, production disruptions, intellectual property infringements, or adverse litigation rulings.

