Life is Rare

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JCR Pharmaceuticals Co., Ltd.

JCR Pharmaceuticals Announces Development Plan in Japan for Givinostat for Duchenne Muscular Dystrophy

Hyogo, Japan – July 15, 2026 – JCR Pharmaceuticals Co., Ltd. (TSE 4552; "JCR"), a global specialty biopharmaceutical company dedicated to developing therapies for rare and genetic diseases, today announced its development plan in Japan for givinostat, a treatment for Duchenne muscular dystrophy (DMD), following consultation with the Pharmaceuticals and Medical Devices Agency (PMDA).

Based on the outcome of the consultation, JCR plans to:

- Submit a marketing authorization application in Japan in 2026, primarily using overseas clinical data
- Obtain approval and launch the treatment in Japan in 2027
- Conduct a separate clinical trial in Japanese patients with DMD to evaluate pharmacokinetics and safety

Givinostat is an oral histone deacetylase inhibitor licensed from Italfarmaco S.p.A. It has already been approved in multiple countries and regions, including the United States, the European Union, and the United Kingdom, for the treatment of DMD in patients aged 6 years and older, in accordance with local prescribing information. As announced in December 2025, JCR holds exclusive rights to develop and commercialize the treatment in Japan. Because its mechanism of action is not dependent on specific dystrophin gene mutations, givinostat has the potential to be used in patients with DMD regardless of underlying mutation type.

"Following our consultation with PMDA, we have established a specific development plan in Japan," said Hiroyuki Sonoda, Ph.D., President and Chief Scientific Officer of JCR. "By using overseas clinical data, we believe this development strategy can help bring this treatment to patients with DMD in Japan more quickly. DMD remains an area of significant unmet medical need, and we will advance development with the goal of obtaining approval and launching it in Japan in 2027."

JCR remains committed to advancing research and development to help meet the expectations of patients and families waiting for effective treatment options.

The impact of this announcement on JCR's consolidated financial results for this fiscal year (ending March 31, 2027) is expected to be minor.

About Duchenne Muscular Dystrophy (DMD)

Duchenne muscular dystrophy (DMD) is a rare, progressive neuromuscular disorder caused by mutations in the dystrophin gene. These mutations prevent the production of functional dystrophin,

causing the dystrophin-associated protein complex (DAPC) to break down. This makes muscle fibres more vulnerable to damage and increases histone deacetylase (HDAC) levels in the muscle cells, blocking the activation of important genes needed for muscle maintenance and repair. As a result, muscle fibres experience ongoing damage, leading to chronic inflammation and poor regeneration. Over time, muscle cells die and are replaced by scar tissue and fat[1-4]. DMD primarily affects males, with symptoms typically appearing between the ages of two and five years. As the condition progresses, muscle weakness worsens, leading to difficulty walking and eventually loss of ambulation. Over time, the heart and respiratory muscles are also affected, which are the leading causes of premature death[5]. DMD is one of the most severe and common forms of childhood muscular dystrophy, with a global birth incidence of approximately 1 in 5,050 boys[6]. DMD affects an estimated 3,500 patients in Japan[7].

About Givinostat

Givinostat (Duvyzat®) was discovered through Italfarmaco's research and development efforts in collaboration with Telethon and Duchenne Parent Project (Italy). Givinostat is an orally administered histone deacetylase (HDAC) inhibitor that regulates the excessive HDAC activity characteristic of DMD muscles. By doing so, it helps restore the expression of key genes and biological processes essential for muscle maintenance and repair. Its mechanism of action is independent of the specific dystrophin gene mutation causing the disease.

About Italfarmaco S.p.A.

Founded in 1938 in Milan, Italy, Italfarmaco is a private global pharmaceutical company that has led the successful development and approval of many pharmaceutical products around the world. The Italfarmaco group has operations in more than 90 countries through directly controlled or affiliated companies. The company is a leader in pharmaceutical research, product development, production and commercialisation with proven success in many therapeutic areas including immuno-oncology, gynaecology, neurology, cardiovascular disease and rare diseases. Italfarmaco's rare disease unit includes programmes in Duchenne muscular dystrophy, Becker muscular dystrophy, amyotrophic lateral sclerosis and polycythaemia vera.

About JCR Pharmaceuticals Co., Ltd.

JCR Pharmaceuticals Co., Ltd. (TSE 4552) is a global specialty pharmaceutical company that develops treatments that go beyond rare diseases to solve the world's most complex healthcare challenges. We continue to build upon our 50-year legacy in Japan while expanding our global footprint into the U.S., Europe, and Latin America. We improve patients' lives by applying our scientific expertise and unique technologies to research, develop, and deliver next-generation therapies. Our approved products in Japan include therapies for the treatment of growth disorder, MPS II (Hunter syndrome), Fabry disease, acute graft-versus host disease, and renal anemia. Our investigational products in development worldwide are aimed at treating rare diseases including MPS I (Hurler, Hurler-Scheie and Scheie syndrome), MPS II, MPS IIIA and B (Sanfilippo syndrome type A and B), and more. Our core values – Putting people first, Forging our own path, Always advancing, and Committed to excellence – mean that the work we do benefits all our stakeholders, including partners, patients and employees. We strive to expand the possibilities for patients while accelerating medical advancement at a global level. For more information, please visit JCR's global website: https://jcrpharm.com/.

Cautionary Statement Regarding Forward-Looking Statements

This document contains forward-looking statements that are subject to known and unknown risks and uncertainties, many of which are outside our control. Forward-looking statements often contain words such as "believe," "estimate," "anticipate," "intend," "plan," "will," "would," "target"

and similar references to future periods. All forward-looking statements regarding our plans, outlook, strategy and future business, financial performance and financial condition are based on judgments derived from the information available to us at this time. Factors or events that could cause our actual results to be materially different from those expressed in our forward-looking statements include, but are not limited to, a deterioration of economic conditions, a change in the legal or governmental system, a delay in launching a new product, impact on competitors' pricing and product strategies, a decline in marketing capabilities relating to our products, manufacturing difficulties or delays, an infringement of our intellectual property rights, an adverse court decision in a significant lawsuit and regulatory actions.

This document involves information on pharmaceutical products (including those under development). However, it is not intended for advertising or providing medical advice. Furthermore, it is intended to provide information on our company and businesses and not to solicit investment in securities we issue.

Except as required by law, we assume no obligation to update these forward-looking statements publicly or to update the factors that could cause actual results to differ materially, even if new information becomes available in the future.

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