Life is Rare

September 28, 2026

JCR Pharmaceuticals Co., Ltd.

JCR Pharmaceuticals Receives Orphan Drug Designation from the Ministry of Health, Labour and Welfare of Japan for Givinostat for Duchenne Muscular Dystrophy

Hyogo, Japan – September 28, 2026 – JCR Pharmaceuticals Co., Ltd. (TSE 4552; "JCR") today announced that the Ministry of Health, Labour and Welfare of Japan granted orphan drug designation to givinostat, an investigational drug for the treatment of Duchenne muscular dystrophy (DMD).

Givinostat (marketed as Duvyzat® in the US, EU, and the UK) is an orally administered histone deacetylase (HDAC) inhibitor developed by Italfarmaco S.p.A. It has been approved in multiple countries and regions, including the US, EU, and the UK, and is prescribed to patients aged 6 years and older with DMD in accordance with local prescribing information. It is currently not approved in Japan.

In December 2025, JCR entered into an exclusive license agreement with Italfarmaco for the development and commercialization of givinostat in Japan. JCR is currently advancing development toward obtaining marketing approval in Japan and plans to submit a marketing authorization application for the product in Japan by the end of 2026. In addition to receiving orphan drug designation in Japan, givinostat has also been granted Priority Examination status.

"The designation of givinostat as both an orphan drug and a product eligible for Priority Examination represents an important milestone toward its commercialization in Japan," said Hiroyuki Sonoda, Ph.D., President and Chief Scientific Officer of JCR Pharmaceuticals. "We remain committed to bringing this new treatment option to patients with DMD in Japan as quickly as possible, addressing the significant unmet medical needs of the DMD community."

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

About Orphan Drug Designation in Japan

Under the orphan drug designation system, the Ministry of Health, Labour and Welfare of Japan takes special measures to support and promote research activities for the development of drugs for rare diseases. Drugs satisfying the following criteria may be designated as orphan drugs: they are targeting a condition with an incidence of fewer than 50,000 patients in Japan, or their intended use is for the treatment of a designated intractable disease. The drugs should be indicated for the treatment of serious diseases including difficult-to-treat diseases for which there are high unmet medical needs, such as there is no approved medicinal product or higher efficacy, or safety is expected compared with an approved medicinal product. Moreover, the applicant for orphan drug designation needs to have enough resources and plans for development of its product as an orphan drug in Japan.

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 commercialization with proven success in multiple therapeutic areas including oncology, gynecology, neurology, cardiovascular disease and rare diseases. Italfarmaco's rare disease unit includes programs in Duchenne muscular dystrophy, Becker muscular dystrophy, amyotrophic lateral sclerosis and polycythemia 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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