



February 28, 2024

JCR Pharmaceuticals Co., Ltd.

JCR Pharmaceuticals Celebrates Rare Disease Day 2024 and Highlights its Commitment to the Rare Disease

Hyogo, Japan, February 28, 2024 -- Thursday, February 29 is Rare Disease Day ("RDD"). RDD activities aim to raise awareness of rare diseases and to improve the quality of life of individuals with rare and intractable diseases through better diagnoses and treatments. [JCR Pharmaceuticals Co., Ltd.](#) (TSE 4552; Chairman and President: Shin Ashida; "JCR") endorses the important mission of RDD and will advocate for RDD 2024 in Japan. Honoring Rare Disease Day is a means for JCR to express its most profound respect for individuals living with rare diseases, their families, caregivers, physicians, and supporters in their network.

There are more than 6,000 rare diseases with an estimated 300 million individuals worldwide living with a rare disease*. Given the rarity of disease conditions affecting a limited number of individuals, viable treatment options and diagnosis opportunities for each disease are limited as well, not to mention the challenge for disease awareness to the public.

"A rare genetic disease is not a choice in lifestyle. Often, it is difficult to understand the challenges in daily living, anxiety, isolation, fears, anger, or hope that individuals and their families undergo as they navigate their diagnostic journey, often with unfulfilled hopes in rare diseases due to no or limited treatment options. At JCR, we believe that the best way to support individuals living with rare diseases is to develop game-changing therapies that help them live a more normal life and fulfill some of their hopes and dreams. Our commitment extends into ultra-rare diseases to do everything to ensure that no one is left behind," explains Shin Ashida, Chairman and President of JCR Pharmaceuticals, as he shares the company's view on rare diseases. "JCR's commitment to the advancement of novel therapies for rare diseases is reflected in a portfolio of 17 development programs for rare and ultra-rare lysosomal storage disorders."

Every year as the last day of February approaches, awareness for RDD and rare diseases is further promoted with RDD posters displayed in the offices and facilities of JCR group companies and official RDD badges worn by JCR members in Japan and overseas. Internal activities such as hosting lectures by patient families and healthcare professionals engaged in rare diseases provide us insights and appreciation for what we do and can do.

Highlights of external awareness raising-activities for RDD:

Japan

- Advocating RDD 2024 in Japan and supporting their awareness-raising activities by displaying posters about rare diseases at Tokyo Tower

The U.S.

- Conducting a presentation for elementary school students about rare lysosomal storage disorders

Brazil

- Joining the *Global Chain of Lights* and light up our office with RDD colors.
- Displaying RDD posters and disseminating RDD information externally.

For JCR, RDD does not stop on the last day of February; it reminds us of our purpose every day to help raise awareness and generate change for the rare disease community worldwide.

About Rare Disease Day (RDD)

Rare Disease Day (RDD), which takes place every year on the last day in February, was established in Europe in 2008 with the aim of raising the quality of life (QOL) of patients around the world with rare or intractable diseases. RDD is now observed in more than 100 nations, and making it the largest social awareness event in this field on a global scale.

About JCR Pharmaceuticals Co., Ltd.

JCR Pharmaceuticals Co., Ltd. (TSE 4552) is a global specialty pharmaceuticals company that is redefining expectations and expanding possibilities for people with rare and genetic diseases worldwide. We continue to build upon our 49-year legacy in Japan while expanding our global footprint into 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. Our approved products in Japan include therapies for the treatment of growth disorder, 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 (Hunter syndrome), MPS III A and B (Sanfilippo syndrome types A and B), and more. JCR strives to expand the possibilities for patients while accelerating medical advancement at a global level. Our core values – reliability, confidence, and persistence – benefit all our stakeholders, including employees, partners, and patients. Together we soar. For more information, please visit <https://www.jcrpharm.co.jp/en/site/en/>.

References

*Rare Disease Day website: <https://www.rarediseaseday.org/>
RDD JAPAN website: <https://rddjapan.info/>
National Organization for Rare Disorders (NORD) website:
<https://rarediseases.org/rare-disease-day/>

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