PRESENTATIONS OVERVIEW
February 2023

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.

Please note, JR-141 (or IZCARGO®) is approved in Japan for the treatment of patients with mucopolysaccharidosis II, or MPS II. JR-141 is under regulatory review in Brazil, and JCR is conducting a Phase 3 clinical trial of JR-141 in the United States, Europe, and Latin America. There are other investigational therapies mentioned within these abstracts that are in development and are not approved for commercial use. Any information on the investigational therapies contained herein is not intended to provide medical advice, nor should it be used as a substitute for the advice provided by your physician or other healthcare providers. Please visit the JCR Pharmaceuticals website for more information.

At WORLD*Symposium*™ 2023, JCR Pharmaceuticals is pleased to present a total of 10 posters focusing on investigational therapies intended to treat lysosomal storage disorders (LSDs).

JR-141: Post-Approval Clinical Trials

Oral Presentation:

Real-world data of enzyme replacement therapy with pabinafusp alfa for neuronopathic MPS-II: updated clinical data from Japan

Presenter: Yoshikatsu Eto, MD

Date and Time: Friday, Feb. 24 from 2:00 - 3:00 PM ET

Session: Clinical Applications

Poster 110:

Real-world data of enzyme replacement therapy with pabinafusp alfa for neuronopathic MPS-II: updated clinical data from Japan

Author: Yoshikatsu Eto, MD

Date and Time: Friday, Feb. 24 from 4:00 - 5:00 PM ET

Session: Clinical Applications: Poster Session VI, Kiosk 2-A

Poster 239:

Changes in quality of life reflecting neurobehavioral improvements observed by caregivers/physicians of patients with neuronopathic mucopolysaccharidosis: an interview-based survey from Brazil following clinical trials with pabinafusp alfa

Author: Ana Maria Martins, MD, PhD, MSc

Date and Time: Friday, Feb. 24 from 3:00 - 4:00 PM ET

Session: Clinical Applications: Poster Session V, Kiosk 27-A

Poster 133:

Long-term neurodevelopmental changes in subjects with MPS-II following long-term treatment with pabinafusp alfa - an integrated analysis from pre- and post-approval clinical trials in Brazil and Japan

Author: Roberto Giugliani, MD, PhD

Date and Time: Friday, Feb. 24 from 4:00 - 5:00 PM ET

Session: Clinical Applications: Poster Session VI, Kiosk 3-A

For more information:

→ **jcrpharm.co.jp** ✉ **ir-info@jp.jcrpharm.com**

AGT-194: Clinical Data

Oral Presentation:

Treatment of CLN1 Disease with a Blood-Brain Barrier Penetrating Lysosomal Enzyme

Presenter: Andreas Hahn, MD, PhD, MSc

Date and Time: Thursday, Feb. 23 from 2:00 - 3:00 PM ET

Session: Translational Research

Poster 153:

Treatment of CLN1 Disease with a Blood-Brain Barrier Penetrating Lysosomal Enzyme

Author: Andreas Hahn, MD, PhD, MSc

Date and Time: Thursday, Feb. 23 from 3:00 - 4:00 PM ET

Session: Translational Research: Poster Session III, Kiosk 14-A

JR-171: Clinical Data

Oral Presentation:

Interim results of a Phase 1/2 study of JR-171 (lepunafusp alfa), a novel brain-penetrant enzyme replacement therapy for MPS I

Presenter: Paul Harmatz, MD

Date and Time: Friday, Feb. 24 from 9:00 - 10:00 AM ET

Session: Clinical Applications

Poster 157:

Interim results of a Phase 1/2 study of JR-171 (lepunafusp alfa), a novel brain-penetrant enzyme replacement therapy for MPS I

Author: Paul Harmatz, MD

Date and Time: Friday, Feb. 24 from 3:00 - 4:00 PM ET

Session: Clinical Applications: Poster Session V, Kiosk 15-A

JR-441: Non-Clinical Data

Oral Presentation:

Nonclinical pharmacodynamics, pharmacokinetics and safety profiles of anti-human transferrin receptor antibody-fused N-sulfoglucosamine sulfohydrolase for mucopolysaccharidosis type IIIA

Presenter: Asuka Inoue

Date and Time: Saturday, Feb. 25 from 8:00 - 9:00 AM ET

Session: Contemporary Forum

Poster 180 (JR-441 non-clinical data):

Nonclinical pharmacodynamics, pharmacokinetics and safety profiles of anti-human transferrin receptor antibody-fused N-sulfoglucosamine sulfohydrolase for Mucopolysaccharidosis IIIA

Author: Asuka Inoue

Date and Time: Saturday, Feb. 25 from 3:00 - 4:00 PM ET

Session: Contemporary Forum: Poster Session VII, Kiosk 24-A

Investigational Therapy for the Treatment of Krabbe Disease (Non-Clinical Data)

Poster 179:

Life-span extension in Krabbe disease mice by treatment with a transferrin receptor-targeted galactocerebrosidase

Author: Atsushi Imakiire

Date and Time: Saturday, Feb. 25 from 3:00 - 4:00 PM ET Session: Contemporary Forum: Poster Session VII, Kiosk 23-A

JR-471: Clinical Data

Poster 154:

International online survey of families' experience of Fucosidosis

Author: Kotaro Hamauchi

Date and Time: Saturday, Feb. 25 from 3:00 - 4:00 PM ET Session: Contemporary Forum: Poster Session VII, Kiosk 15-B

JR-471: Non-Clinical Data

Poster 381:

A fusion protein of anti-human transferrin receptor antibody and alfa-L-fucosidase 1 is a prospective candidate for the treatment of fucosidosis

Author: Eiji Yoden

Date and Time: Saturday, Feb. 25 from 4:00 - 5:00 PM ET

Session: Contemporary Forum: Poster Session VIII, Kiosk 23-A

JR-141: Non-Clinical Data

Poster 247:

Intravenous treatment with pabinafusp alfa dose-dependently prevents neurological impairment and bone deformities in a mouse model of Mucopolysaccharidosis II

Author: Hideto Morimoto

Date and Time: Saturday, Feb. 25 from 4:00 - 5:00 PM ET

Session: Contemporary Forum: Poster Session VIII, Kiosk 5-A

For more information:

→ jcrpharm.co.jp ir-info@jp.jcrpharm.com

INDICATION

IZCARGO® is indicated for the treatment of mucopolysaccharidosis type II (MPS II), which is also known as Hunter syndrome. IZCARGO® is approved in Japan only.

PRECAUTIONS RELATED TO EFFICACY OR EFFECT

To consider administration to patients who may need to improve in or suppress progression of central nervous system symptoms.

CONTRAINDICATION

IZCARGO® is contraindicated in patients with a history of anaphylactic shock to its any components.

WARNINGS AND PRECAUTIONS

Warnings

Since serious anaphylaxis and shock may occur with use of IZCARGO®, adequate emergency measures should be made ready for execution before initiation of administration, and the patient should be closely monitored during and after the administration. If a serious infusion associated reaction (IAR) occurs, administration of IZCARGO® should be discontinued, and appropriate actions should be taken.

When IZCARGO® is administered to patients with severe respiratory failure or acute respiratory disease, an IAR may lead to acute exacerbation of symptoms. Patient's condition should be closely monitored, and appropriate actions should be taken as needed.

Precautions for Use

IZCARGO® is a protein medicinal product and may cause anaphylactic shock, for which close monitoring is required. If any signs of anaphylaxis are noted, discontinue the infusion, and take appropriate actions. Considering the onset of such symptoms, emergency measures should be made ready for execution.

IZCARGO® may cause IARs such as headache, chills, syncope, fatigue, dizziness, pyrexia, rash, erythema, urticaria, or other symptoms. If an IAR occurs, reduce the rate or temporarily discontinue the infusion, and initiate appropriate drug treatment (e.g., corticosteroids, antihistamines, antipyretic analgesics, anti-inflammatory drugs) or emergency procedures (e.g., oxygen administration, securing of airway, adrenaline administration). Premedication with antihistamines, corticosteroids, etc. should be considered for the subsequent infusion of IZCARGO®.

ADVERSE REACTIONS:

The most commonly reported adverse reactions were pyrexia and urticaria.

For more information:

→ **jcrpharm.co.jp** **ir-info@jp.jcrpharm.com**