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Press Release

March 30, 2026

Tanabe Pharma Announces Positive Data from Phase 3 INSPIRE Study of Dersimelagon in EPP and XLP

- First Oral Therapy to achieve Phase 3 Primary endpoint in EPP and XLP for adults and adolescents
- Statistically significant and clinically meaningful outcomes demonstrated across primary and secondary endpoints
- Dersimelagon was generally well-tolerated, with a safety profile consistent with its mechanism of action.
- Data presented in late-breaking oral session at 2026 American Academy of Dermatology Annual Meeting
- Dersimelagon has been granted both U.S. FDA Fast Track Designation and Orphan Drug Designation. NDA preparation is in progress.

Tanabe Pharma Corporation (Head Office: Chuo-ku, Osaka; Representative Director, CEO: Akihisa Harada, hereinafter "Tanabe Pharma") today announced detailed results from the Phase 3 INSPIRE study of investigational oral dersimelagon in people living with erythropoietic protoporphyria (EPP) and X-linked protoporphyria (XLP). The data were presented in a late-breaking oral session at the 2026 American Academy of Dermatology Annual Meeting on Mar 28, 2026.

Dersimelagon, a selective agonist of melanocortin 1 receptor (MC1R), met the primary endpoint, showing a statistically significant prolongation of average daily sunlight exposure time to first prodromal symptom during Weeks 12–16 versus placebo, with a placebo-adjusted least-squares mean difference of 23.19 minutes (p=0.004) in primary analysis. At Week 16, the effect increased to 29.64 minutes (p=0.004) in supplementary analysis.

Key secondary endpoints were also met, including statistically significant differences in Patient Global Impression of Change (PGIC) and reduction of total pain events compared to placebo (PGIC: -1.83, p<0.001; reduction of total pain events: 39%, p=0.004), supporting clinical meaningful outcome of primary endpoint.

The most common adverse events, occurring more frequently than placebo, were melanocytic nevi (benign moles), headache, nausea, diarrhea, and skin hyperpigmentation, with the mechanism of MC1R agonist. The INSPIRE study enrolled 165 adults and

adolescents aged 12 to 75 years with EPP or XLP. [Dersimelagon has been studied in 410 participants with EPP or XLP to date].

“These Phase 3 results show a clinical improvement in light tolerance before experiencing pain, in study participants with EPP and XLP,” said Amy Yeung, MD, MSc, co-founder and co-director of the Porphyria Center at Massachusetts General Hospital. “People living with EPP and XLP struggle with light intolerance every day. These results suggest the potential for a new treatment option for them.”

EPP and XLP are debilitating, lifelong genetic disorders of the heme biosynthetic pathway characterized by severe, painful phototoxic reactions after exposure to sunlight and some forms of artificial light. Symptoms can occur within minutes of exposure, often beginning in childhood, severely restricting patients’ ability to participate in school, work, and daily activities. Treatment options remain limited for adults, and there are no approved therapies for adolescents.

“Today’s INSPIRE presentation marks an important milestone for people living with EPP and XLP, who continue to face a profound daily burden and significant unmet medical need,” said Akihisa Harada, Chief Executive Officer of Tanabe Pharma Corporation. “dersimelagon is the first oral therapy to demonstrate Phase 3 clinical functional benefit in EPP and XLP, and, if approved, it has the potential to become the first oral treatment option. With the positive data from Phase 3 INSPIRE study, we look forward to advancing dersimelagon toward a planned NDA submission”.

Dersimelagon has been granted both U.S. FDA Fast Track Designation and Orphan Drug Designation, recognizing the significant unmet need and the potential for meaningful therapeutic benefit. Tanabe Pharma has initiated preparations for a New Drug Application (NDA) rolling submission.

*Japan Intractable Diseases Information Center website: https://www.nanbyou.or.jp/entry/5546

Media inquiries:
Japan: kouhou-ml.jp@ml.tanabe-pharma.com
U.S.: Media_TPA.US@mb.tanabe-pharma.com

■ About Dersimelagon (MT-7117)

Dersimelagon was internally discovered and developed by Tanabe Pharma, a Bain Capital portfolio company. Dersimelagon is a novel synthetic, orally-administered, non-peptide small molecule, which acts as a selective agonist of MC1R with a potential for being

effective to increase pain free light exposure in people living with EPP and XLP. Tanabe Pharma is developing dersimelagon for the treatment of EPP or XLP. Dersimelagon is an investigational medication and not approved by FDA or any other regulatory authority. Tanabe Pharma received Fast Track Designation for dersimelagon by the U.S. Food and Drug Administration in June 2018.

■ About INSPIRE Study

The INSPIRE study is a global, randomized, double-blind, placebo-controlled phase 3 clinical trial in adult and adolescent participants with EPP or XLP. Participants were randomly assigned to either a placebo group or an active treatment group (dersimelagon 200 mg once daily) for a 16-week double-blind period, followed by a 36-week open-label extension period of active treatment. The trial, mainly conducted by Tanabe Pharma America, Inc. (Jersey City, New Jersey, USA), enrolled 165 participants with EPP or XLP (aged 12 years and older and under 75 years). The double-blind treatment phase has been completed, while the open-label phase is ongoing.

■About Erythropoietic Protoporphyria and X-Linked Protoporphyria

EPP is an inherited disorder of the heme biosynthetic pathway that results from mutations of the ferrochelatase (FECH) gene or, less commonly XLP that results from mutations in the aminolevulinic acid synthase-2 (ALAS2) gene. Both EPP and XLP are characterized by accumulation of protoporphyrin in blood, erythrocytes and tissues and cutaneous photosensitivity. EPP and XLP usually present early in childhood with extremely painful phototoxic reactions which are preceded by a “prodrome” of tingling, stinging, and/or burning of sun-exposed skin. The onset of prodromal symptoms after direct sun exposure varies but may occur in less than 10 minutes. Importantly, continued exposure to sunlight following the onset of prodromal symptoms will lead to phototoxicity-induced pain.