Media Release
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Breakthrough Therapy Designation granted by the FDA for daridorexant in pediatric insomnia disorder

The FDA recognizes through this designation both the clinical evidence supporting daridorexant and the significant unmet medical need in pediatric insomnia, where no FDA-approved pharmacological treatments are currently available.

The positive large Phase 2 study demonstrated a statistically significant dose-dependent improvement in total sleep time in the overall pediatric population, with pronounced efficacy in patients with neurodevelopmental disorders

This designation enables intensive FDA guidance and more frequent interactions on an efficient development program for daridorexant in pediatric insomnia disorder

Allschwil, Switzerland – November 04, 2026
Idorsia Ltd (SIX: IDIA) today announced that the US Food and Drug Administration (FDA) has granted Breakthrough Therapy Designation to daridorexant for the treatment of insomnia disorder in patients 10 to <18 years of age.

The designation provides for intensive FDA guidance and closer collaboration on the development of daridorexant in children and adolescents. Daridorexant is a next-generation dual orexin receptor antagonist (DORA) that selectively reduces wake drive rather than broadly inhibiting brain activity. Idorsia designed daridorexant with an optimized pharmacokinetic profile to provide efficacy throughout the night while minimizing residual exposure the following morning. This differentiated profile has been associated with improvements in sleep parameters and daytime functioning in adults with insomnia disorder and may be particularly relevant for children and adolescents.

Pediatric insomnia: a substantial burden on children's nights and development

Pediatric insomnia is characterized by difficulty initiating or maintaining sleep that is perceived as a problem by the child or/and caregiver due to its severity, chronicity, frequency, and the significant daytime impairment it causes for the child and family. The prevalence (~8-12 %) of the disorder increases from childhood to adolescence. Its burden is particularly high in those with neurodevelopmental disorders, including autism spectrum disorder and attention-deficit/hyperactivity disorder. Insufficient or disrupted sleep can affect attention, learning, emotional regulation, behavior and quality of life, while worsening the symptoms and comorbidities associated with neurodevelopmental disorders. Despite this burden, no pharmacological treatment is currently approved by the FDA for insomnia disorder in pediatric patients.

Breakthrough Therapy Designation recognizes the high unmet need and positive preliminary clinical evidence

Breakthrough Therapy Designation aims to expedite the development and review of drugs intended to treat a serious condition based on preliminary clinical evidence indicating that an investigational medicine may provide a substantial improvement on a clinically significant endpoint, together with the available safety data. It therefore represents important FDA recognition of the clinical results generated with daridorexant to date, and supports the conduct of further studies required to establish its efficacy and safety for potential approval. This closer collaboration is intended to make the development and review of daridorexant as efficient as possible.

Amer Joseph, MD, Chief Medical Officer and Head of Global Clinical Development commented: "This Breakthrough Therapy Designation underscores both the significant unmet medical need faced by children and adolescents living with insomnia disorder and the encouraging clinical evidence generated with daridorexant to date. Daridorexant was specifically designed with an optimized pharmacokinetic and tolerability profile, allowing it to be largely cleared by the next morning and avoiding accumulation with repeated dosing. This profile enabled us to evaluate multiple doses in our pediatric Phase 2 study, including the highest approved adult dose of 50mg. At this dose, daridorexant demonstrated robust efficacy while maintaining a favorable tolerability profile, an especially important consideration in children and adolescents. We look forward to working closely with the FDA as we prepare the Phase 3 program and generate the evidence needed to bring what could become the first FDA-approved pharmacological treatment for pediatric insomnia disorder to patients and their families."

Next steps in pediatric insomnia

The manuscript describing the full dataset is currently under evaluation with a high-impact scientific journal. In parallel, the team is consolidating supportive comments from global health authorities on the protocol for the pediatric Phase 3 clinical development program. The company expects to initiate the new program in the second half of next year.

Daridorexant for pediatric use is investigational and is not approved or marketed for children or adolescents in any country.

Notes to the editor

About daridorexant – a different kind of insomnia treatment

Insomnia affects millions worldwide, yet commonly used treatments can lead to next day drowsiness, dependence, and withdrawal symptoms – leaving a clear unmet need for safer, effective alternatives.

Discovered by Idorsia, daridorexant works differently from traditional hypnotics. As a dual orexin receptor antagonist, it selectively targets the orexin system, helping to regulate overactive wake signaling without broadly suppressing brain activity. Its optimized pharmacokinetics promote restorative sleep throughout the entire night, reducing morning sleepiness and improving daytime functioning.

Clinical trials published in The Lancet Neurology demonstrated that daridorexant significantly improved sleep onset, sleep maintenance, and self-reported total sleep time in adult patients with insomnia at 25mg and 50mg doses compared to placebo. Daridorexant 50 mg also demonstrated a significant improvement in daytime functioning compared to placebo.

About pediatric insomnia

Pediatric insomnia is characterized by difficulty initiating or maintaining sleep that is perceived as a problem by the child or caregiver due to its severity, chronicity, frequency, and the significant daytime impairment it causes for the child or the family.

About the pediatric Phase 2 study (NCT05423717)

A recording of Professor Carmen Schröder's presentation, featuring the pediatric Phase 2 study results presented at Sleep Europe 2026, is available here: Watch the presentation
The multicenter, randomized, double-blind, placebo-controlled Phase 2 dose-finding study evaluated daridorexant 10, 25 and 50 mg, or placebo, over two weeks in 165 children and adolescents aged 10 to under 18 years with insomnia disorder.
The primary objective of the Phase 2 study was to characterize the dose-response relationship of daridorexant on objective total sleep time (TST), using polysomnography (PSG). Patients were randomized in a 1:1:1:1 ratio to 10, 25, or 50 mg daridorexant. The primary endpoint was the change from baseline to Day 1 in TST measured by PSG. Additional assessments included objective and subjective sleep measures, sleep quality, morning sleepiness and alertness, daytime functioning, pharmacokinetics, safety and tolerability.

The study included children and adolescents with insomnia disorder without neurodevelopmental disorders, participants with subthreshold autism spectrum disorder or attention-deficit/hyperactivity disorder traits, and participants with diagnosed neurodevelopmental disorders.

Analysis of the primary endpoint demonstrated a statistically significant (p=0.0185) dose-dependent improvement in TST from baseline on Day 1, accompanied by additional dose-dependent improvements across multiple objective and subjective sleep parameters. A single 50 mg dose increased sleep time by 50 minutes overall and by 64 minutes in children and adolescents with neurodevelopmental disorders, with a numerical decrease of morning sleepiness.

Importantly, the study confirmed the excellent safety and tolerability profile of daridorexant for the first time in pediatric patients as young as ten. Even at 50 mg – the recommended adult dose – daridorexant's safety profile remained similar to placebo, with no adverse events denoting drug abuse during treatment and no indication of withdrawal symptoms upon treatment discontinuation.

About Idorsia

The purpose of Idorsia is to discover, develop and commercialize innovative medicines to help more patients. To achieve this, we will develop Idorsia into a leading biopharmaceutical company, with a strong scientific core.

Headquartered near Basel, Switzerland – a European biotech hub – Idorsia has a highly experienced team of dedicated professionals, covering all disciplines from bench to bedside; QUVIVIQ™ (daridorexant), a different kind of insomnia treatment with the potential to revolutionize this mounting public health concern; strong partners to maximize the value of our portfolio; a promising in-house development pipeline; and a specialized drug discovery engine focused on small-molecule drugs that can change the treatment paradigm for many patients. Idorsia is listed on the SIX Swiss Exchange (ticker symbol: IDIA).

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The above information contains certain "forward-looking statements", relating to the company's business, which can be identified by the use of forward-looking terminology such as "intend", "estimates", "believes", "expects", "may", "are expected to", "will", "will continue", "should", "would be", "seeks", "pending" or "anticipates" or similar expressions, or by discussions of strategy, plans or intentions. Such statements include descriptions of the company's investment and research and development programs, business development activities and anticipated expenditures in connection therewith, descriptions of new products expected to be introduced by the company and anticipated customer demand for such products and products in the company's existing portfolio. Such statements reflect the current views of the company with respect to future events and are subject to certain risks, uncertainties and assumptions. Many factors could cause the actual results, performance or achievements of the company to be materially different from any future results, performances or achievements that may be expressed or implied by such forward-looking statements. Should one or more of these risks or uncertainties materialize, or should underlying assumptions prove incorrect, actual results may vary materially from those described herein as anticipated, believed, estimated or expected. Idorsia has transferred its rights for aprocitentan, cenerimod and selatogrel to Idorsia Investments SARL to allow the repayment of notes issued in connection with the repurchase offer completed in August 2025. More details on the transfer can be found in the current Financial Report from Idorsia Investments SARL.