PRESS RELEASE

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Saniona receives positive EU orphan drug designation opinion for SAN2668 in CHD2-related neurodevelopmental disorder

Saniona (OMX: SANION), a clinical-stage biopharmaceutical company focused on neurological and psychiatric disorders, today announces that the Committee for Orphan Medicinal Products (COMP) of the European Medicines Agency (EMA) has adopted a positive opinion recommending orphan medicinal product designation for SAN2668 for the treatment of CHD2-related neurodevelopmental disorder (CHD2-RND). This is the first positive COMP opinion for an orphan medicinal product designation for CHD2-RND in the EU. The opinion has been forwarded to the European Commission for a final decision.

CHD2-RND is a rare genetic developmental and epileptic encephalopathy (DEE) caused by changes in the CHD2 gene. Seizures typically begin during the first years of life. Most affected individuals remain refractory to treatment with multiple anti-seizure medications, often in combination. There are currently no treatments specifically approved for CHD2-RND [1].

“The positive COMP opinion is an important regulatory milestone for SAN2668 and supports our strategy to develop the program for severe developmental and epileptic encephalopathies,” said Pierandrea Muglia, Saniona Chief Medical Officer. “There is a significant unmet medical need and a strong preclinical rationale for SAN2668 in CHD2-related epilepsy. Orphan drug designation would facilitate regulatory support as we advance SAN2668 towards clinical development.”

Orphan medicinal product designation in the EU supports development of medicines for life-threatening or chronically debilitating rare conditions. Designated medicines may benefit from incentives including protocol assistance from EMA, reduced regulatory fees and eligibility for ten years of market exclusivity following marketing authorization in the EU.

CHD2-RND represents a specific indication within Saniona’s broader development strategy for SAN2668 in DEEs. Generalized spike-and-wave discharges are characteristic features of CHD2-related epilepsy [1,2] providing a rationale for SAN2668’s α3-driven mechanism.

More about CHD2-related neurodevelopmental disorder (CHD2-RND)
CHD2-RND is caused by pathogenic variants in the CHD2 gene. Most cases arise de novo, meaning the variant is not inherited from a parent [2]. The disorder is characterized by early-onset epileptic encephalopathy, with refractory seizures and cognitive slowing or regression associated with frequent epileptiform activity [1]. Typical seizure types include drop attacks, myoclonic seizures and atonic-myoclonic-absence seizures, with generalized spike-wave discharges on EEG. Many patients are sensitive to flashing lights [1,2]. Most affected individuals have intellectual disability or developmental delay, and autism is common [2].

More about SAN2668
SAN2668, Saniona’s lead program, is a GABA-A α2/α3 positive allosteric modulator designed for severe pediatric epilepsies. It selectively potentiates GABA-A α3 and α2 receptors while maintaining balanced α1 modulation and modest α5 activity. This profile is designed to preserve benzodiazepine-class anti-seizure efficacy while minimizing adverse effects.

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Strong α3 modulation targets thalamocortical circuits implicated in spike-wave EEG pathology, while robust α2 activity is intended to provide protection against focal and generalized convulsive seizures. SAN2668 is designed to offer a wider therapeutic window, allowing higher receptor occupancy than can be tolerated with non-selective benzodiazepines. Its preclinical profile supports its potential to become a best-in-class therapy for pediatric epilepsy.

[1] Carvill GL, Mefford HC. CHD2-Related Neurodevelopmental Disorders. GeneReviews®. 2015 (updated 2021)
[2] Puri AG et al. Seizure 2025;132:88–97

About Saniona
Saniona is a clinical-stage biopharmaceutical company focused on discovering, developing, and delivering innovative treatments for neurological and psychiatric disorders. The company's internal pipeline includes SAN2668 for pediatric epilepsy syndromes and SAN2465 for major depressive disorder. Saniona has established strategic collaborations with leading pharmaceutical companies, including Jazz Pharmaceuticals, which holds global rights to SAN2355 for epilepsy, Acadia Pharmaceuticals, which holds worldwide rights to ACP-711 for essential tremor, and with Medix, which holds rights to tesofensine for obesity in Mexico, and Argentina. Saniona's ion channel discovery platform is further validated through research collaborations with Boehringer Ingelheim, Cephagenix and AstronauTx, which holds worldwide rights to ATX0926. Headquartered in Copenhagen, Saniona is listed on the Nasdaq Stockholm Main Market.
For more information, visit **www.saniona.com**.

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Attachments
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