THX Pharma (Theranexus) and Exeltis secure EMA approval of the paediatric investigation plan (PIP) for TX01, unlocking the PUMA registration pathway

Approval of the PIP by the EMA's Paediatric Committee (PDCO) clears a critical regulatory gate, paving the way for a Paediatric Use Marketing Authorisation (PUMA) submission and securing up to 10 years of European market protection for TX01 in Niemann-Pick type C disease

Lyon, France – Madrid, Spain – June 9, 2026 – 6:00 pm CEST – THX Pharma (Theranexus), a pharmaceutical company specializing in treatments for rare neurological diseases, and Exeltis, a global pharmaceutical company within the Insud Pharma group, announce that the European Medicines Agency (EMA), through its Paediatric Committee (PDCO), has issued a positive Opinion on the Paediatric Investigation Plan (PIP) for TX01 in Niemann-Pick type C disease. The EMA's subsequent Decision confirms the alignment of TX01 development programme with European paediatric regulatory standards and unlocks a Paediatric Use Marketing Authorisation (PUMA) registration pathway in the European Union. Application will be filed by Exeltis in Q1 2027.

This achievement is a major regulatory milestone in the partnership between THX Pharma and Exeltis, building directly on the successful completion of the first key milestone announced on October 14, 2025, and confirms that both companies remain on track to deliver TX01 to patients with Niemann-Pick type C disease as planned.

A decisive regulatory gate for TX01

The PIP is the regulatory instrument governing paediatric development in the European Union. The PDCO's positive Opinion confirms that the agreed quality, non-clinical and clinical development package, including the proposed paediatric formulation strategy of TX01, is appropriate to support the targeted indications and age subsets, and that the timing and design of the studies are acceptable to the Agency.

Crucially, an approved PIP is a regulatory prerequisite for any PUMA submission. With this Decision, THX Pharma and Exeltis now have a clear and de-risked path to file a centralised PUMA application with the EMA, which, once granted, would enable a single marketing authorisation valid across all 27 EU Member States plus Iceland, Liechtenstein and Norway.

Mathieu Charvériat, Chairman and Chief Executive Officer of THX Pharma, commented: *"Securing the PDCO's positive Opinion on the PIP is one of the most discriminating regulatory milestones a paediatric drug development programme can achieve in Europe. It validates the scientific and clinical foundations of TX01 and, equally importantly, opens the PUMA pathway – a uniquely well-suited regulatory framework that combines a focused dossier, a centralised procedure and up to 10 years of paediatric market protection. With this decision in hand, THX Pharma and Exeltis are fully on track to deliver TX01 to European patients as planned, and we confirm our objective of commercial launch in Europe in 2027."*

The PUMA: A tailored pathway for high-value paediatric innovation

The PUMA is a dedicated marketing authorisation route created by the EU Paediatric Regulation for medicines developed exclusively for paediatric use, based on active substances already authorised in adults.

For TX01, it offers a uniquely favourable combination of regulatory and commercial advantages:

- **Up to 10 years of paediatric market protection**, comparable in scope to orphan exclusivity,
- **A centralised EU procedure** delivering a single authorisation, one harmonised SmPC and label across the 27 EU Member States plus Iceland, Liechtenstein and Norway, and streamlined pricing & reimbursement submissions,
- **A reinforced position in P&R (price and reimbursement) negotiations**, as European HTA bodies increasingly recognise dedicated paediatric formulations and PUMA status as a differentiating value driver in ultra-rare indications.

A shared commitment to patients with Niemann-Pick Type C diseases

TX01 is a novel paediatric formulation of an already approved compound, developed to address the specific needs of children living with Gaucher disease and Niemann-Pick type C disease – two rare, progressive and severely debilitating lysosomal storage disorders for which the paediatric therapeutic options remain limited. Today's announcement reinforces the strategic rationale of the THX Pharma–Exeltis partnership and confirms the timeline towards commercialisation in Europe as early as 2027, followed by launches in Latin America and selected Middle Eastern markets.

About THX Pharma

THX Pharma (Theranexus) is a pharmaceutical company specializing in treatments for rare neurological diseases. Its first drug, TX01, is expected to be commercialized soon — particularly in Europe by Exeltis, but also in the United States, Canada, and Australia — for Niemann-Pick type C disease and Gaucher disease. Its second drug, Batten-1, targets the juvenile form of Batten disease and could become the first approved therapy for this condition. THX Pharma also has an innovative antisense oligonucleotide platform, codeveloped with leading research laboratories, dedicated to rare neurological diseases. THX Pharma, a trade name of Theranexus, is listed on Euronext Growth Paris (FR0013286259 – ALTHX).

For more information: http://www.thxpharma.com - Follow us on LinkedIn

About the Insud Pharma Group

Insud Pharma is a pharmaceutical group with a history spanning over 45 years, present in around 50 countries, with 10,000 employees worldwide and 20 production sites. Insud Pharma operates across the entire pharmaceutical industry value chain, offering its expertise in scientific research, development, production, sales, registration and marketing of a wide range of active pharmaceutical ingredients (APIs), finished dosage forms (FDFs), and branded and innovative medicines for human and veterinary care, as well as biopharmaceutical products. Insud Pharma is committed to improving global health by providing accessible, effective, safe, and high-quality treatments through its various business units, with continuous investment in R&D and the latest technologies. Through its brand Exeltis, Insud Pharma holds a leading position in women's health and is a key player in Central Nervous System therapies.

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Disclaimer

This press release contains forward-looking statements relating to THX Pharma (Theranexus) and its activities, including its prospects and product development. THX Pharma believes that these forward-looking statements are based on reasonable assumptions. However, forward-looking statements are not guarantees of future performance, as they relate to future events and depend on circumstances that may or may not occur in the future, and on various risks and uncertainties, including those described in the universal registration document filed by the company with the AMF (Autorité des Marchés Financiers) on April 29, 2026, under number D.26-0321, a copy of which is available on the company's website (www.thxpharma.com), and on changes in the economic situation.