

Spain: EIB provides Minoryx with up to €25 million to support development of breakthrough therapies in orphan neurodegenerative diseases



©Getty Images

- **EIB support to develop breakthrough treatments to address orphan neurodegenerative diseases with high unmet medical needs**
- **Focus on Minoryx's lead asset, leriglitzone, a novel, disease-modifying PPAR- γ agonist**
- **Venture debt operation supported by the Investment Plan for Europe**

The European Investment Bank (EIB) today announced that it has approved €25 million financing for Minoryx Therapeutics, a Phase 3 clinical stage biotech company focused on the development of differentiating treatment options for orphan central nervous system (CNS) disorders.

The EU bank will grant long-term financing to Minoryx to drive the company's research activities in orphan genetic diseases for which there are currently no approved drugs available. The EIB investments will specifically support the development of Minoryx's leriglitzone, a disease-modifying treatment (PPAR- γ agonist) currently being evaluated in three late-stage clinical trials:

- Pivotal Phase 2/3 ADVANCE study in adrenomyeloneuropathy (AMN), a disease that constitutes the chronic form of X-ALD (X-linked adrenoleukodystrophy) with onset in adulthood and characterised by progressive neurodegeneration of the spinal cord, resulting in progressive motor dysfunction.
- Phase 2 NEXUS study in cerebral adrenoleukodystrophy (cALD), the most aggressive form of X-ALD, typically affecting children between 2 and 12 years of age and characterised by brain inflammation, leading to rapid cognitive decline and death.
- Phase 2 FRAMES study in Friedreich's Ataxia (FRDA), a life threatening disease characterised by neurodegeneration resulting in loss of coordination, muscle strength, and cardiomyopathy.

Estimates indicate that the investment in this research, development and innovation (RDI) project will help create over 50 jobs during the implementation phase.

EIB Vice-President [Ricardo Mourinho Félix](#), who is responsible for the Bank's operations in [Spain](#), said: *"We are very happy to sign an agreement that reaffirms the EIB's commitment to fostering innovation in the healthcare sector by driving the development of new treatments that will have a positive impact on the quality of life of people affected by rare diseases of the central nervous system. Our support for the research programme of Minoryx, a Spanish biotech company, will also help to improve the competitiveness of the European pharmaceutical industry and create highly skilled jobs."*

European Commissioner for the Economy, Paolo Gentiloni, said: *"This EU support will help Minoryx to develop breakthrough therapies for genetic diseases and treatments for diseases of the central nervous system. The coronavirus pandemic has shown how important it is to continue to push scientific boundaries and deliver medication for rare diseases. The European Commission will continue to support companies' efforts in this sense at every opportunity."*

*"The EIB brings Minoryx valuable non-dilutive financial resources, alongside our strong and supportive shareholder base, which, we believe, is a clear endorsement of the potential of leriglitzone to improve the lives of patients living with debilitating neurodegenerative disorders", said **Didier Le Normand, Group CFO and General Manager Belgium**. "The current clinical studies with leriglitzone continue to advance as planned and we remain on track to report top line data from the pivotal ADVANCE study before the end of the year."*

The EIB will provide funds for this RDI project by way of a venture debt operation under the [European Fund for Strategic Investments \(EFSI\)](#), a financing instrument used by the EU bank to assist leading companies in innovative research sectors. Since it was launched by the EIB under the Juncker Plan in 2016, this initiative has granted over €2 billion in financing for projects in sectors such as robotics, artificial intelligence and biomedicine. EIB venture debt financing targets European companies with up to 3 000 employees in the fields of biotechnology and health sciences, software and ICT, engineering and automation, and renewable energy and clean

technology.

Background information

About EIB venture debt and Investment Plan for Europe

The EIB's venture debt product is a financing instrument that supports startup and fast-growing innovative companies in cutting-edge technology sectors. It combines the advantages of a long-term loan with a remuneration model based on the company's performance. Venture debt transactions help strengthen the borrower's economic capital without diluting the shares of existing investors. The product, developed four years ago in response to market needs, is backed by the European Fund for Strategic Investments (EFSI), the financial pillar of the Investment Plan for Europe. The European Fund for Strategic Investments (EFSI) is the main pillar of the [Investment Plan for Europe](#). It provides first-loss guarantees enabling the EIB to invest in more and often riskier projects. The projects and agreements approved for financing under EFSI have mobilised €535.4 billion in investment so far, a quarter of which is supporting research, development and innovation projects.

About Minoryx

Minoryx is a clinical stage biotech company focusing on the development of novel therapies for orphan CNS diseases with high unmet medical needs. The company's lead programme, leriglitazone (MIN-102), a novel, selective PPAR γ agonist, is currently being evaluated in X-ALD and Friedreich's Ataxia. The company is backed by a syndicate of experienced investors, which includes Caixa Capital Risc, Roche Venture Fund, Ysios Capital, Kurma Partners, Fund+, Chiesi Ventures, S.R.I.W, Idinvest, SFPI-FPIM, HealthEquity and Sambrinvest, and has support from a network of other organisations. Minoryx was founded in 2011, has operations in Spain and Belgium and has so far raised more than €60 million.

About leriglitazone

Leriglitazone (MIN-102) is a novel bioavailable and selective PPAR- γ agonist with a potential best-in-class profile indicated for CNS diseases. It has a demonstrated sufficient brain penetration and favourable safety profile. It showed robust preclinical proof-of-concept in animal models of multiple diseases by modulating pathways leading to mitochondrial dysfunction, oxidative stress, neuroinflammation, demyelination and axonal degeneration. Leriglitazone has successfully completed a phase 1 clinical trial showing good safety, tolerability and CNS engagement of PPAR- γ receptors at levels equivalent to those required for efficacy in preclinical models. Leriglitazone has the potential to treat several CNS disorders, including orphan diseases, and is currently being evaluated in a registration enabling Phase 2/3 study in AMN, a registration enabling Phase 2 in cALD and in a Phase 2 in Friedreich's Ataxia.

About X-ALD

X-ALD (X-linked adrenoleukodystrophy) is an orphan neurodegenerative disease.

AMN and cALD are the two most common phenotypes of X-ALD, which account for 45% and 35% respectively. The global incidence of X-ALD is approximately 6.2/100 000 live births.

The age of onset of cALD patients is typically 4-8 years old. Untreated patients progress quickly, as severe neurological function impairment appears 6-24 months after disease onset, leading to early death in 2-4 years.

AMN is characterised by progressive spastic paraparesis, sensory dysfunction and incontinence. This form progresses chronically with onset of symptoms typically in adulthood and poor prognosis.

There is currently no therapeutic treatment available for X-ALD. Several observational studies have demonstrated that hematopoietic stem cell transplantation (HSCT) may improve the five-year overall survival for cALD patients. However, there is no evidence that HSCT improves clinical outcomes of patients with AMN.