



Urania Therapeutics closes a €3.5M initial seed round

Combining venture capital and capabilities in kind for drug development, this innovative operation will help fund the development of promising readthrough compounds for the advent of new drugs targeting monogenic diseases, a further step towards precision medicine

Strasbourg, France, November 6, 2019 – Urania Therapeutics, a biopharmaceutical company specializing in the identification and development of 'readthrough' compounds, today announces it has successfully raised €3.53M (\$3.89M) in seed funding to unlock the therapeutic potential of ribosome-based treatments.

The seed round, led by Advent France Biotechnology, will allow Urania Therapeutics to consolidate its capabilities and optimize its proprietary compounds, enabling it to select drug candidates for further progress. This ambitious life science project combines venture capital from Advent France Biotechnology (AFB) and Cap Innov'Est (CIE) and strong capabilities, provided by NovAliX, a CRO specialized in medicinal chemistry and biophysics for drug development.

Urania Therapeutics is researching new therapeutic options for a wide range of monogenic diseases, by targeting the decoding centre of the human ribosome, thereby restoring production of functional protein. This will pave the way for new treatments for patients with rare monogenic diseases, such as Duchenne Muscular Dystrophy (DMD) and Cystic Fibrosis (CF), as well as certain cancer subtypes caused by nonsense mutations.

The readthrough approach has the potential to transform the current standard of care for orphan diseases, many of which have no treatments available. Since readthrough compounds target a gene-independent mechanism, this approach could lead to a generic treatment for patients with these life-threatening conditions. [According to analysts from Evaluate Pharma](#), orphan drugs represent a growing segment of the pharmaceutical market and are expected to almost double by 2024, reaching \$239bn (€217bn).

Urania Therapeutics' breakthrough approach relies on the seminal work and expertise of its scientific founders, world-leading ribosome structure experts Marat Yusupov and Gulnara Yusupova, CNRS research directors at the Institute of Genetics and Molecular and Cellular Biology (IGBMC) in Strasbourg, France.

"At Advent France Biotechnology (AFB), we strive to seed fund bold science with breakthrough therapeutic outcomes. With Urania Therapeutics, we have combined promising ribosome science – a strong biophysics-based development platform – with entrepreneurial acumen. Strasbourg is a centre for high-profile academic science and biotech entrepreneurship," said Alain Huriez, chairman and managing partner, AFB.

"It is a real pleasure to see this project, which we followed from its inception, spread its wings today. We are convinced that Urania Therapeutics combines all the ingredients to bring to the patients efficient therapeutic compounds based on readthrough mechanisms," said Jean-François Rax, investment director, CIE.

"Teaming up with seasoned venture capital firms, AFB and CIE, to turn scientific advances into valuable drug candidates is an excellent initiative that will help us explore new therapeutic avenues," said Denis Zeyer, CEO, NovAliX.



"This new syndicate, led by high-profile life science investors and a CRO with strong proficiency in biophysical approaches, is an innovative combination," said Jean-Paul Renaud, founder, president and CSO of Urania Therapeutics. "The new partnership will help us move towards the successful translation of structural understanding of ribosome and discover breakthrough drugs for uncured genetic disorders."

The funding will also enable Urania Therapeutics to look at the translation from bench to bedside, from atomic-scale mechanistic understanding to discovering new drugs for unmet medical needs. Urania Therapeutics aims to have an in vivo proof of concept for the first undisclosed therapeutic indications within 18 months.

About Advent France Biotechnology

Advent France Biotechnology (AFB) is an AMF-regulated company created in 2016. The team, managed by Alain Huriez and Matthieu Coutet, includes professionals with extensive scientific, medical and operational experience, as well as a long-standing track record of entrepreneurial and investment successes across Europe. AFB invests in a range of sectors within the life sciences, specifically in drug discovery and new medical technologies.

www.adventfb.com

About NovAliX

NovAliX is a drug discovery-focused CRO with several unique technologies. It is highly proficient in chemistry and biophysics.

The company has set up one of the world's most comprehensive biophysics platforms, from screening to identification and detailed characterization of drug-target interactions using protein X-ray crystallography, native mass spectrometry, nuclear magnetic resonance (NMR), surface plasmon resonance (SPR) and cryo-electron microscopy.

NovAliX' innovative, cost-effective, collaborative and co-located research programs involve dedicated teams of scientists working throughout Europe in its partners' laboratories. Close proximity fosters the seamless transfer of knowledge in both directions and speeds up the research process.

NovAliX, founded in 2002, is based in Strasbourg, France, in the heart of the BioValley Upper Rhine region, France.

www.novalix.com

About Cap Innov'Est

Cap Innov'Est is a €45M (\$49.6M) French regional seed fund (Grand Est, Bourgogne-Franche-Comté) dedicated to investing in young, innovative startups. Cap Innov'Est was launched in July 2014 and has already invested in 25 startups. Cap Innov'Est is managed by Capital Grand Est with its partner, Invest PME (Siparex group). Cap Innov'Est is supported by Fonds National d'Amorçage (National Seed Fund), Regions Grand Est and Bourgogne-Franche-Comté, SAFIDI, Caisse d'Epargne d'Alsace and Bourgogne/Franche-Comté, BNP Paribas and CIFIC.

www.capitalgrandest.eu

About Urania Therapeutics

Urania Therapeutics is a privately held French biopharmaceutical company specializing in the identification and development of 'readthrough' compounds following a structure-based-drug design approach (SBDD). These compounds induce the overcoming of premature stop codons during protein synthesis to allow the production of missing full-length protein.

In restoring production of functional protein by targeting the decoding centre of the human ribosome, the aim is to provide new therapeutic options for a vast array of monogenic diseases, such as Duchenne Muscular Dystrophy (DMD) and Cystic Fibrosis (CF), as well as



certain cancer subtypes caused by nonsense mutations, a new illustration of precision medicine.

Urania Therapeutics' approach relies on the work and expertise of its scientific founders, world-leading ribosome structure experts Marat Yusupov and Gulnara Yusupova, CNRS research directors at IGBMC (Institute of Genetics and Molecular and Cellular Biology).

Headquartered in Strasbourg (France), the company was created in 2015.

www.uraniatx.com

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