

April 11, 2022



Poxel Announces PXL770 Awarded FDA Fast Track Designation for X-linked Adrenoleukodystrophy

LYON, France--(BUSINESS WIRE)-- [POXEL SA](#) (Euronext: POXEL - FR0012432516), a clinical stage biopharmaceutical company developing innovative treatments for chronic serious diseases with metabolic pathophysiology, including non-alcoholic steatohepatitis (NASH) and rare metabolic disorders, is pleased to announce that the U.S. Food and Drug Administration (FDA) has granted Fast Track Designation (FTD) to PXL770 for the treatment of patients with adrenomyeloneuropathy (AMN), the most common form of X-linked adrenoleukodystrophy (ALD). PXL770 is a novel, first-in-class direct adenosine monophosphate-activated protein kinase (AMPK) activator that is preparing to enter into a Phase 2a clinical Proof-of-Concept (POC) biomarker study midyear, subject to additional financing.

Poxel CEO, Thomas Kuhn, commented: "Having PXL770 awarded Fast Track Designation by the FDA soon after our other promising product, PXL065, is a strong recognition of the potential of both our programs in adrenoleukodystrophy, a significant unmet medical need. Poxel aims at developing innovative treatments to improve the life of patients with serious and rare chronic diseases with metabolic pathophysiology, and the Fast Track Designation could offer PXL770 the ability to substantially accelerate the approval timeline in ALD. We are preparing the next steps to initiate our two Phase 2a clinical studies midyear, with results anticipated early 2023."

Fast Track Designation (FTD)

- FTD is designed to expedite development of pharmaceutical products which demonstrate the potential to address unmet medical needs in serious or life-threatening conditions.
- FTD provides Poxel with substantially enhanced access to FDA, including opportunities for face-to-face meetings and written consultations throughout the remaining development of PXL770.
- Drugs with FTD are eligible to apply for Accelerated Approval and Priority Review at the time of a New Drug Application (NDA) submission, which may result in faster product approval.
- FTD also allows for 'rolling review', whereby Poxel may submit completed sections of the NDA as they become available, rather than at the end of development.

Next Steps

The Phase 2a clinical POC biomarker studies for Poxel's two products, PXL770 and PXL065, in X-linked ALD are, subject to additional financing, anticipated to begin midyear, with results anticipated early 2023.

Fast Track Designation

Introduced under the FDA Modernization Act (1997), Fast Track Designation (FTD) may be awarded by the FDA to investigational drugs which treat a serious or life-threatening condition, and which fill an unmet medical need. Filling an unmet medical need is defined as providing a therapy where none exists or providing a therapy which may be potentially better than available therapy. The FDA notes that 'the purpose of the Fast Track program is to get important new drugs to the patient earlier¹'. FTD must be requested by the sponsor company and must be accompanied by a detailed review of both preclinical and clinical data.

The key benefits of FTD comprise enhanced access to the FDA, with regular and more frequent opportunities for consultation and discussion. In addition, drugs with FTD may be eligible for Accelerated Approval, in which a new medicine is approved prior to the availability of definitive data, and Priority Review, in which the standard 10-month review process is reduced to six months. Drugs with FTD may also enter a 'rolling review' of their NDA submission, in which sections are submitted and reviewed as they become available, substantially expediting the approval process.

About ALD

X-linked adrenoleukodystrophy (ALD) is an orphan neurometabolic disease caused by mutations in the ABCD1 gene which encodes for a key protein that is required for metabolism of very long chain fatty acids (VLCFA) by peroxisomes (cellular organelles). ALD is the most common leukodystrophy with a prevalence similar to hemophilia – up to 1/10,000 individuals in the general population have ALD [<https://rarediseases.org>]. Forms of this disease include cerebral ALD (C-ALD) and adrenomyeloneuropathy (AMN) which is the most common form – typically occurring in adolescence through adulthood. AMN is characterized by chronic and progressive distal axonopathy involving the long tracts of the spinal cord and to a lesser extent the peripheral nerves resulting in progressive stiffness and weakness in the legs, impaired gait and balance, incontinence, and loss of sensation. Nearly all men with a diagnosis of ALD will develop AMN, and many women also present with features of AMN with a later onset. C-ALD is characterized by inflammatory demyelination of cells in the brain and typically afflicts children, but many men with AMN may also develop cerebral disease; these white matter brain lesions lead to severe neurologic deficits and death. There are no approved medicines for ALD (other than glucocorticoid supplements for associated adrenal insufficiency). C-ALD when first detected in early childhood, can be treated with hematopoietic stem cell transplantation. HSCT is currently limited to early stage of C-ALD and this procedure is at risk of severe adverse reactions.

About Poxel SA

Poxel is a **clinical stage biopharmaceutical company** developing **innovative treatments for chronic serious diseases with metabolic pathophysiology**, including **non-alcoholic steatohepatitis (NASH)** and rare disorders. Poxel has clinical and earlier-stage programs from its adenosine monophosphate-activated protein kinase (AMPK) activator and deuterated TZD platforms targeting chronic and rare metabolic diseases. For the treatment

of NASH, **PXL065** (deuterium-stabilized *R*-pioglitazone) is in a streamlined Phase 2 trial (DESTINY-1). **PXL770**, a first-in-class direct AMPK activator, has successfully completed a Phase 2a proof-of-concept trial for the treatment of NASH, which met its objectives. For the rare inherited metabolic disorder, adrenoleukodystrophy (ALD), the company intends to initiate Phase 2a proof of concept studies with PXL065 and PXL770 in patients with adrenomyeloneuropathy (AMN). **TWYMEEG®** (Imeglimin), Poxel's first-in-class lead product that targets mitochondrial dysfunction, has been approved and launched for the treatment of type 2 diabetes in Japan. Poxel expects to receive royalties and sales-based payments from Sumitomo Pharma. Poxel has a strategic partnership with Sumitomo Pharma for Imeglimin in Japan, China, South Korea, Taiwan and nine other Southeast Asian countries. The Company intends to generate further growth through strategic partnerships and pipeline development. Listed on Euronext Paris, Poxel is headquartered in Lyon, France, and has subsidiaries in Boston, MA, and Tokyo, Japan.

For more information, please visit: www.poxelpharma.com

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¹ For more information on the Fast Track Designation, see: <https://www.fda.gov/patients/fast-track-breakthrough-therapy-accelerated-approval-priority-review/fast-track>

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Source: Poxel SA