



ADARx Pharmaceuticals Announces Positive Interim Phase 1 Clinical Data for its Novel siRNA Targeting Complement Factor B (CFB)

November 7, 2025

– Single subcutaneous dose of ADX-038 achieved near-complete alternative pathway suppression for 6 months, supporting potent and selective CFB silencing –

– Phase 2 studies are ongoing to assess the efficacy and safety of ADX-038 in patients with complement-mediated diseases –

SAN DIEGO, Calif. – November 7, 2025 – [ADARx Pharmaceuticals, Inc.](#) (ADARx), a late-stage clinical biotechnology company developing next-generation RNA therapeutics, today announced positive interim Phase 1 data for ADX-038, an investigational small interfering RNA (siRNA) therapeutic candidate that targets complement factor B (CFB). ADX-038 is being developed to treat multiple diseases that are driven by activation of the alternative pathway (AP) of the complement system, including renal diseases (immunoglobulin A nephropathy (IgAN) and complement 3 glomerulopathy (C3G)).

The data presented at the American Society of Nephrology (ASN) Kidney Week 2025 demonstrated that a single subcutaneous dose of ADX-038 was well tolerated and led to deep and sustained reduction of circulating plasma CFB protein levels, which resulted in selective and maximal suppression of complement AP activity without changes in the complement system classical pathway (CP) activity. This selective AP inhibition may reduce the risk of infection caused by therapeutics that also inhibit the CP.

"These clinical data on potency, selectivity and durability support the potential of semi-annual dosing to treat IgAN and C3G," said Zhen Li, Ph.D., President and Chief Executive Officer of ADARx. "For patients who must cycle through multiple therapies to control their disease, a treatment that keeps the alternate pathway turned off for months could meaningfully reduce kidney damage and clinic visits."

Dr. Li added, "ADX-038 was shown to inhibit alternative-pathway activity and represents a true pipeline-in-a-product opportunity. As such, we are pursuing multiple indications for ADX-038 including across renal, hematologic and ocular diseases."

Interim Phase 1 Data Summary

The Phase 1 study evaluated the safety, tolerability, pharmacokinetics and pharmacodynamics of ADX-038 in healthy adult participants (ADX-038 n=31; placebo n=10). The poster presentation summarized the effects of ADX-038 on complement AP and CP activity in healthy participants. Clinical safety was based on adverse events (AEs), monitoring of clinical lab tests, and physical exams.

After a single 6mg/kg subcutaneous dose of ADX-038:

- Near-complete AP inhibition (up to 99.6%) was sustained through Day 180.
- CP activity was unchanged, suggesting preservation of a regulated immune response to reduce the risk of encapsulated bacterial infections.
- ADX-038 was well tolerated with the most common AEs being headache and upper respiratory tract infection.

The positive Phase 1 data could support a differentiated therapeutic profile for ADX-038 with regards to both the duration and depth of AP activity inhibition in treating complement-mediated diseases.

About ADX-038

ADX-038 is an investigational siRNA therapy designed to selectively cleave complement factor B (CFB), a validated target in the complement pathway. The complement system is a key component of the immune system that enhances, or complements, the activity of both adaptive and innate immune responses. Dysregulation of this system has been implicated in a broad range of autoimmune and inflammatory diseases. As such, ADX-038 offers the potential to serve as a pipeline-in-a-product, addressing multiple indications through a single therapeutic approach. Multiple Phase 2 clinical trials have been initiated to evaluate the efficacy and safety of ADX-038 in patients with complement-mediated diseases including renal diseases (immunoglobulin A nephropathy (IgAN) and complement 3 glomerulopathy (C3G), geographic atrophy (GA) secondary to age-related macular degeneration (AMD), and paroxysmal nocturnal hemoglobinuria (PNH).

About ADARx Pharmaceuticals

[ADARx Pharmaceuticals, Inc.](#) is a late-stage clinical biotechnology company dedicated to transforming cutting-edge science into next-generation RNA medicines across a broad range of therapeutic areas. We have developed proprietary technology to control the expression of specific disease drivers with highly selective RNA targeted therapies with the goal of delivering life-changing treatments for patients with urgent unmet medical needs. ADARx is focused on advancing and expanding a deep pipeline of highly potent, durable and selective RNA-targeted therapeutic candidates, developing product candidates for the treatment of complement-mediated, genetic, cardiovascular, thrombotic, central nervous system and metabolic (obesity) diseases. In addition to our wholly-owned programs, we have entered into a collaboration and license option agreement with AbbVie to develop siRNA therapeutics across multiple disease areas, including neuroscience, immunology and oncology. Follow ADARx on [LinkedIn](#).

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