

Press Release

One04 Therapeutics launches on encouraging CPV-104 Phase 1 interim results and plans to expand clinical development to include kidney diseases and dry AMD

- **CPV-104 interim Phase 1 results support favorable safety and tolerability, with encouraging early efficacy trends in patients with C3 glomerulopathy**
- **Detailed interim Phase 1 data to be released in a virtual event after ASN Kidney Week**
- **One04 is fully funded to key value inflection points, data from two CPV-104 clinical trials, both starting in early 2027:**
 - **Phase 2 to treat complement-mediated kidney diseases**
 - **Phase 1 to treat dry age-related macular degeneration/geographic atrophy**

Freiburg, Germany, September 15, 2026 – One04 Therapeutics GmbH & Co. KG today announced its launch as a clinical-stage biopharmaceutical company with encouraging Phase 1 results for CPV-104, a first-in-class, full-length recombinant human Factor H candidate with disease-modifying potential across a broad range of complement-mediated diseases.

The launch comes at an important stage in the development of CPV-104: The company will present detailed interim Phase 1 data in patients with C3 glomerulopathy (C3G) on October 26, 2026, after the American Society of Nephrology (ASN) Kidney Week conference, followed by advancement into Phase 2 development in complement-mediated kidney diseases and expansion of Phase 1 clinical development to treat dry age-related macular degeneration (AMD), including geographic atrophy (GA), in early 2027.

“CPV-104 is a pipeline-in-a-product that has matured into a highly promising clinical candidate for complement-mediated diseases,” said **Björn Cochlovius, Ph.D., Chief Executive Officer of One04 Therapeutics**. “The formation of One04 provides the dedicated structure and funding needed to accelerate its development. Our current funding enables us to reach multiple value inflection points, including an initial Phase 2 readout in complement-mediated kidney disease and Phase 1 data in the much larger indication of dry AMD/GA.”

One04 Therapeutics has received undisclosed funding from D11Z.Ventures, a leading European venture capital fund specializing in early-stage investments. CPV-104 was discovered and developed using Eleva Biologics’ moss-based Bryotechnology production platform. The asset was transferred to One04 Therapeutics to facilitate optimal operational focus and strategic independence for both companies.

In contrast to current approaches, which inhibit individual components of the complement cascade, CPV-104 is designed to restore the system’s endogenous regulation through Factor H. This

differentiated approach is intended to control pathological complement overactivation while preserving broader complement function important for homeostasis, host protection and immune defense. Preclinical and early interim clinical findings support this therapeutic rationale, which will be further investigated in continued clinical development.

Top-line interim results to be presented in October from the first two cohorts of the multiple-ascending dose (MAD) portion of the ongoing Phase 1 trial show favorable safety, tolerability and pharmacokinetics for CPV-104, alongside early efficacy trends in patients with C3G. The MAD portion includes three dose cohorts totaling 18 patients, of which one is ongoing.

The placebo-controlled single-ascending dose (SAD) portion was completed in October 2025 and evaluated CPV-104 across four dose cohorts in 21 healthy volunteers, with no safety concerns observed.

Detailed interim Phase 1 data will be presented in greater detail via livestream on October 26, 2026, following One04 Therapeutics' attendance at ASN Kidney Week. CPV-104 has EU Orphan Designation for C3G.

"The encouraging interim Phase 1 data underscore the potential of CPV-104 as a differentiated therapeutic candidate in C3G, an indication with significant unmet need," said **Erich Rajkovic, Ph.D., MBA, Chief Business Officer of One04 Therapeutics**. "We believe this potential extends to a broader range of complement-mediated diseases of the kidney and eye, supported by CPV-104's differentiated approach to restoring endogenous complement regulation from pro-inflammatory toward immune homeostasis. We look forward to several important clinical and strategic value inflection points this year and next."

One04 Therapeutics will initiate an open-label Phase 2 study in early 2027 to evaluate CPV-104 in up to 24 patients with C3G and selected other complement-mediated kidney diseases. Eligible patients who completed the Phase 1 C3G study may be offered the opportunity to continue treatment in the Phase 2 study.

One04 also plans to initiate an open-label Phase 1 study of CPV-104 in dry AMD and its advanced form GA in early 2027. A subsequent, larger, open-label, comparator-controlled Phase 2 study in complement-mediated kidney diseases is planned for late 2027, subject to additional financing.

ABOUT ONE04 THERAPEUTICS

One04 Therapeutics is a clinical-stage biopharmaceutical company developing CPV-104, a first-in-class, recombinant, full-length human Factor H therapy. Factor H has been widely researched and has broad potential to treat complement-mediated diseases of the kidney and eye. Early CPV-104 Phase 1 clinical data showed good initial safety, tolerability and early efficacy trends in patients with a complement-mediated kidney disease. Factor H is known to restore endogenous complement regulation from pro-inflammatory toward immune homeostasis, suggesting CPV-104 has disease-modifying potential.

For regular updates about One04 Therapeutics, visit www.one04.com or follow us on [LinkedIn](#).

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