INV501 in cancer

INV501: A novel potent oral small molecule to enhance anti-tumor activation of the immune system

Programm / Ausschreibung	Life Sciences, Life Sciences, Life Science Ausschreibung 2023	**Status**	abgeschlossen
Projektstart	01.10.2023	**Projektende**	31.01.2026
Zeitraum	2023 - 2026	**Projektlaufzeit**	28 Monate
Keywords	novel small molecule, cancer therapy, immuno-oncology, solid tumors, breast cancer		

Projektbeschreibung

Trotz neuer Therapiemöglichkeiten ist die Behandlung von soliden Tumoren nach wie vor eine globale Herausforderung. Die Aufgabe eines funktionierenden Immunsystems besteht darin Tumorzellen zu eliminieren, was jedoch bei Krebspatienten unterbleibt. Mit einer niedermolekulare Substanz, INV501, beschreitet invIOs neue Wege, indem es eine spezifische anti-Tumor-Immunantwort mittels spezialisierter Immunzellen sogenannten T-Zellen auslöst. Studien an Mäusen zeigen, dass die orale Verabreichung von INV501 gut verträglich ist und zur Bildung aktivierter T-Zellen führt, die die Tumore erkennen und dauerhaft zerstören. Bei schwer behandelbaren humanen Erkrankungen wie dem “Triple-negative” Brustkrebs (TNBC) und auch anderen Tumoren mit hoher Tumorlast, könnte INV501 verabreicht als patientenfreundliche Pille, die Tricks der Krebszellen, dem Immunsystem zu entkommen, wieder rückgängig machen. Das Projekt umfasst die Translation der präklinischen Daten in einen finalen Medikament-Kandidaten mit günstigem pharmakologischem Profil, die Aufsetzung eines Herstellprozesses, toxikologische Studien und 3D-Modellierung von humanen Indikationen in sogenannten Tumoroiden aus frischen Tumorproben. Das für Krebspatienten wegweisende Behandlungskonzept soll nach Abschluss der Arbeiten in eine klinische Studie übergeleitet werden.

Endberichtkurzfassung

The project successfully achieved its overarching goal of advancing a first-in-class anti-cancer drug candidate (INV501) from a pool of leads into a robust preclinical candidate ready for first-in-human studies. All major milestones defined at the outset were completed, and the program delivered a well-balanced molecule with promising efficacy and safety characteristics. While the project timeline was extended from March 2025 to January 2026, this was primarily due to scientifically justified decisions—such as the delayed selection of the final candidate to allow for a more comprehensive data package—as well as technical challenges in manufacturing and limited availability of animals for GLP toxicology studies.

From a scientific perspective, the project generated strong preclinical evidence supporting the therapeutic potential of the candidate. Lead optimization and candidate selection were fully completed, and efficacy was demonstrated across multiple tumor models, including lung, melanoma, breast, colon, and renal cancers. Toxicology studies progressed substantially, with most milestones achieved; the rat GLP toxicology study has been completed, while the dog study remains to be finalized.

Overall, the preclinical work package reached approximately 97% completion, reflecting a high level of maturity of the program.

On the manufacturing and CMC side, the project successfully established a scalable production process. A CDMO partner (Patheon) was identified and engaged, and a novel synthesis route for the compound was developed despite its chemical complexity. This enabled the production of material suitable for GLP toxicology studies, alongside the development of release assays and initiation of stability testing. All milestones in this work package were achieved, marking full completion.

In terms of execution and strategic positioning, the project demonstrated strong operational performance. All planned milestones were delivered, key external partners were secured, and intellectual property was significantly strengthened through the filing of two patent applications—one already published and the other receiving a highly favorable early assessment. This represents a notable value inflection point for the program.

Beyond its technical achievements, the project also highlights meaningful long-term impact. The development of an orally available small-molecule therapy introduces a potentially transformative alternative to existing immunotherapies, which are often based on complex biologics or cell therapies. This approach offers advantages in manufacturing efficiency, eliminates the need for cold-chain logistics, and enables at-home treatment, thereby reducing both environmental footprint and healthcare system burden.

Overall, the project delivered a clinically relevant drug candidate with strong preclinical validation, robust manufacturing readiness, and a solid IP position, laying the foundation for transition into clinical development.

Projektpartner

- invIOs GmbH