JLPHealth

FOR IMMEDIATE RELEASE

Press Release: Achilles heel of glioblastoma. JLP Health and its partners discover combinatorial treatment option for incurable brain tumors.

By studying the mechanism of action from a widely used Nobel Prize winning anti-malaria drug named Artemisinin, the authors were able to pharmacologically sensitize brain cancer cells to this natural substance and selectively eliminate brain tumors in numerous relevant model systems.

Vienna, Austria, 7 February 2023 - JLP Health GmbH, a privately held biotechnology company developing innovative treatments for cancer and infectious diseases, rationally designed a novel and highly effective treatment strategy to selectively kill incurable brain tumors. The study was conducted in collaboration with researchers from the Institute of Molecular Biotechnology of the Austrian Academy of Sciences (IMBA) and the Medical University of Vienna (both Vienna, Austria) and is published in EMBO Molecular Medicine.

The discovery of the natural substance Artemisinin as an anti-malaria treatment from Traditional Chinese Medicines saved millions of lives and was awarded with the Nobel Prize in Physiology in 2015. Besides its anti-malaria activity, Artemisinin exerts cell toxicity and is therefore investigated in several cancer studies, however the molecular mechanism of action remained largely elusive.

Using unique forward genetic screens in mammalian haploid cells, the current study identifies the heme biosynthesis pathway essential for Artemisinin's cancer cell toxicity. Boosting heme biosynthesis via the bottleneck metabolite 5-ALA then allowed the team of scientists and clinicians to sensitize patient-derived glioblastoma cells to Artemisinin killing. Importantly, the synergistic anti-cancer activities of 5-ALA and Artemisinin extend to other highly relevant in vitro and in vivo human brain tumor models.

Co-author and lead JLP Health Scientist Michael Orthofer said: "This combinatorial treatment showed striking effects on cancer tumor burden in multiple models, from tumor cells in classic tissue culture, to therapy resistant tumor spheroids from patients, engineered brain tumors in stem cell derived human brain organoids, and, importantly patient-derived brain tumors implanted into the mouse brain. In all these cases, our combination therapy selectively killed the tumor cells while sparing the surrounding normal brain cells. These results are highly encouraging to move this candidate treatment into clinical trials."

"The survival rates for patients with glioblastoma are dismal, hardly anything works, making it paramount to develop new treatment options for patients", adds Josef Penninger, senior author of the study and Chairman of the Board for JLP Health. "The breakthrough came when we realized that 5-ALA is already clinically approved as imaging agent and millions of people with malaria are treated with Artemisinin today, allowing us to translate our findings very rapidly to clinical trials in humans. It is remarkable that 5-ALA specifically marks malignant glioblastoma cells for surgical resection by inducing heme biosynthesis preferentially in tumor tissue. For patients, this adds a critical layer of specificity to Artemisinin's cancer killing activity."

JLP Health is currently working with researchers and oncologists at the Medical University of Vienna to advance this novel therapy to clinical studies.

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To learn more about the study "A whole-genome scan for Artemisinin cytotoxicity reveals a novel therapy for human brain tumors" published on February 06, 2023, in the Journal EMBO Molecular Medicine. refer to DOI: https://doi.org/10.15252/emmm.202216959

About Glioblastoma

Glioblastoma multiforme is the most aggressive and most common malignant primary brain tumor in humans with a steadily increasing incidence of currently about 3-4 in 100,000 people[1]. Despite major advances in treatment, including surgical resection followed by radiotherapy and temozolomide chemotherapy as current standard of care, virtually all patients relapse with a medium survival period of only 15 months[2]. With an average onset of 64 years, glioblastoma belongs to the cancers with the greatest years of potential life lost[3]. Therefore, novel treatment opportunities are desperately needed.

About JLP Health

JLP Health is a privately held biotech company based in Vienna, Austria, focused on the discovery of new drug target structures to develop innovative therapies for diseases with high unmet medical need. JLP Health has developed unique and unbiased screening approaches at unprecedented single amino resolution (structural biology by genetics) to identify molecular modes of action required for the activity of anti-cancer drugs. Besides the oncology focus, this platform technology is applied to uncover host cell factors essential for viral infections. Moreover, JLP Health continues to uncover molecular mechanisms of natural substances via its genetic screening capabilities with the goal to develop rational treatment options based on fundamental understanding of drug actions.

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2. Stupp, R., et al. "High-grade malignant glioma: ESMO Clinical Practice Guidelines for diagnosis, treatment and follow-up." Annals of oncology 21 (2010): v190-v193. DOI: https://doi.org/10.1093/annonc/mdq187

3. Rouse, Chaturia, et al. "Years of potential life lost for brain and CNS tumors relative to other cancers in adults in the United States, 2010." Neuro-oncology 18.1 (2015): 70-77. DOI: https://doi.org/10.1093/neuonc/nov249

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FORWARD LOOKING STATEMENTS

Information set forth in this press release contains forward-looking statements, which involve a number of risks and uncertainties. The forward-looking statements contained herein represent the judgement of JLP Health as of the date of this press release. Such forward-looking statements are neither promises nor guarantees but are subject to a variety of risks and uncertainties, many of which are beyond our control, and which could cause actual results to differ materially from those contemplated in these forward-looking statements. JLP Health assumes no obligation to update forward-looking statements contained in this release as the result of new information or future events or developments.

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