





PRESS RELEASE

ISCIII and IDP Pharma researchers validate efficacy of a new molecule to treat glioblastoma

- A team of scientists at Carlos III Health Institute (ISCIII), in collaboration with researchers from Hospital 12 de Octubre in Madrid and biotechnology firm IDP Pharma -based at the Barcelona Science Park- have published a study in the journal Neurotherapeutics revealing that IDP-410, a new experimental drug, curbs the growth of glioblastoma in animal models. The researchers hope to get new data to help prove its efficacy in clinical trials in the future.
- The study provides promising data for the development of treatments targeting the N-MYC protein and suggest that IDP-410 -designed and developed by IDP Pharma- can inhibit this protein and stop the appearance and development of glioblastoma, the most common and aggressive brain tumour, with a survival rate of 15-20 months and highly resistant to current treatments.
- This public-private partnership project promotes synergies between the research experience in brain tumours of the Neurooncology Unit at ISCIII and the Multidisciplinary Neurooncology Unit at Hospital 12 de Octubre, and the technology and know-how of IDP Pharma in the design and discovery of first-in-class drugs for a new class of therapeutic target: IDP proteins (Intrinsically Disordered Proteins).

Barcelona, 2 February 2022. A team of scientists at the Carlos III Health Institute (ISCIII), in collaboration with researchers at Hospital 12 de Octubre in Madrid and biotechnology firm IDP Pharma, based at the Barcelona Science Park, has published in the journal Neurotherapeutics a paper presenting new findings regarding the development, evolution and treatment of glioblastomas, the most common brain tumours for which there is no effective treatment.

The main authors of the paper are Pilar Gómez-Sánchez, Ricardo Gargini and Berta Segura-Collar, who are part of Gliomalab -an interdisciplinary team of researchers from the ISCIII Neurooncology Unit and the Multidisciplinary Neurooncology Unit (UMNO) at Hospital 12 de Octubre- and Laura Nevola, co-founder and CSO/COO of IDP Pharma.

Glioblastomas are very aggressive tumours that are resistant to treatments developed so far, meaning patients' prognosis, treated with surgery, radiation and chemotherapy, is complicated and the average life expectancy is 15 to 20 months.

The results of this study are another step towards the possibility of developing a new therapeutic option for glioblastoma. On the one hand, the research has confirmed that glioblastomas express high levels of the protein N-MYC, a transcription factor involved in brain development that, when deregulated, promotes the activation of genes related to tumour progression. On the other hand, it also showed that its function can be modulated specifically and directly with the new experimental drug developed by IDP Pharma, IDP-410.







"We're pleased with the results of this collaboration, which shows the great therapeutic potential of these new targets in severe indications like glioblastomas. The possibility of modulating the function of these proteins, normally considered 'undruggable', using drugs designed specifically for them, like IDP-410, has been and remains a goal of IDP Pharma. This study shows that our strategy opens the door to future development of therapeutic options for patients," explains Laura Nevola, co-founder and CSO/COO of IDP Pharma.

The research team has shown that IDP-410 curbs the growth of glioblastomas implanted in animals' brains, reaching the N-MYC protein in tumour tissue, and reducing its vascularisation, meaning the ability to create the blood vessels needed to grow. Researchers highlight that the possible relationship between N-MYC function and the expression of mesenchymal and angiogenic genes is one of the key elements of the process. They hope to get new data to help prove its efficacy in clinical trials in the future.

"Given that this type of tumour is highly aggressive, developing drugs like IDP-410, which stop both the growth of tumour cells and the creation of new blood cells to feed the tumour, opens up a promising path for providing effective therapies for patients with glioblastoma," explains **Pilar Sánchez-Gómez**, head researcher in the ISCIII Neurooncology Unit.

The results of the study provide new data for developing treatments targeting the N-MYC protein and suggest that experimental drug IDP-410 could become the first effective treatment specifically for N-MYC and possibly applicable for treating glioblastoma. The study also confirms that, to treat this type of tumour, it is key to affect not only tumour cell proliferation and survival, but also the interplay between these cells and the cell microenvironment where the cancer develops.

• Paper reference: Gargini, R., Segura-Collar, B., Garranzo-Asensio, M. et al. "IDP-410: a Novel Therapeutic Peptide that Alters N-MYC Stability and Reduces Angiogenesis and Tumor Progression in Glioblastomas". Neurotherapeutics (2022). https://doi.org/10.1007/s13311-021-01176-6.

■ About IDP Pharma

IDP Pharma is a biotechnology company founded in 2015 by Dr Santiago Esteban and Dr Laura Nevola, experts in novel therapeutic targets and drug design. The biotechnology firm, based in the Barcelona Science Park, focuses on developing novel therapies to treat cancer. Its unique strategy is based on developing first-in-class drugs targeting a new class of therapeutic targets: IDP proteins (Intrinsically Disordered Proteins). Developing drugs for new therapeutic targets makes it possible to bring new treatments to the market, which is particularly significant in diseases currently without any therapeutic

More information [+]

■ About Gliomalab

Gliomalab is an interdisciplinary team of researchers led by Dr Pilar Gómez-Sánchez, Dr Ricardo Gargini and Dr Juan Sepúlveda that is working to decode the behaviour of one of the most aggressive types of cancer, gliomas. To do so, the Neurooncology Unit (Carlos III Health Institute - ISCIII) and the Multidisciplinary Neurooncology Unit (UMNO, Hospital 12 de Octubre) have joined forces, which allows them to work on some of the basic aspects of glioma biology, carry out preclinical studies, and do more patient-orientated research. More information [+]

More information:

Azucena Berea • Press Officer • Barcelona Science Park • 93 403 46 62 •aberea@pcb.ub.cat

José A. Plaza • Head of Communications del ISCIII • 91 822 21 96 / 669 18 73 84 • ja.plaza@isciii.es