

Glioblastoma Window-of-Opportunity update

October 12, 2026

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Oncopeptides is a global biotech company focused on research and development of therapies for difficult-to-treat hematological diseases. The company uses its proprietary Peptide Drug Candidate platform, PDC, to develop compounds that rapidly and selectively deliver cytotoxic agents into cancer cells. Pepaxti® (melphalan flufenamide, also called melflufen) has been granted Marketing Authorization, in the European Union, the EEA-countries Iceland, Lichtenstein and Norway, as well as the UK. Pepaxti is indicated in combination with dexamethasone for the treatment of adult patients with multiple myeloma who have received at least three prior lines of therapies, whose disease is refractory to at least one proteasome inhibitor, one immunomodulatory agent, and one anti-CD38 monoclonal antibody, and who have demonstrated disease progression on or after the last therapy. For patients with a prior autologous stem cell transplantation, the time to progression should be at least 3 years from transplantation. Melflufen was granted an accelerated approval in the US in February 2021, under the trade name Pepaxto®. The product is currently not marketed in the US.

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Presenter



Sofia Heigis
Chief Executive Officer



Henrik Bergentoft
Chief Financial Officer

What was announced?



Confirmed Blood-Brain Barrier (BBB) penetration

Initial patient data from the ongoing OP-701 (INSULA) Window-of-Opportunity study demonstrates that Oncopeptides' proprietary PDC platform successfully crosses the human blood-brain barrier in glioblastoma patients.



Pharmacological concentrations in patient tissue

Biopsies from resected tumor tissue showed consistent presence of the active compound, reaching concentrations aligned with preclinical models and expectations.



Consistent safety profile

Treatment was well tolerated in the evaluated patients, with safety findings consistent with prior clinical experience and no unexpected surgical complications reported.

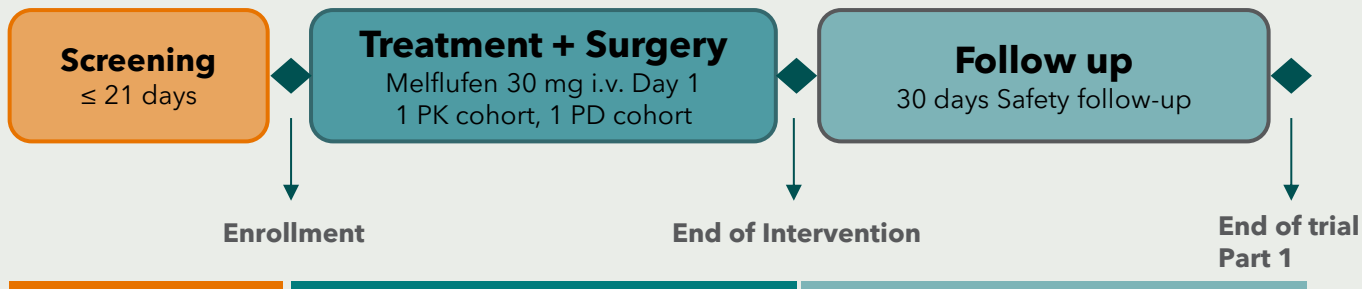


Platform validation in CNS tumors

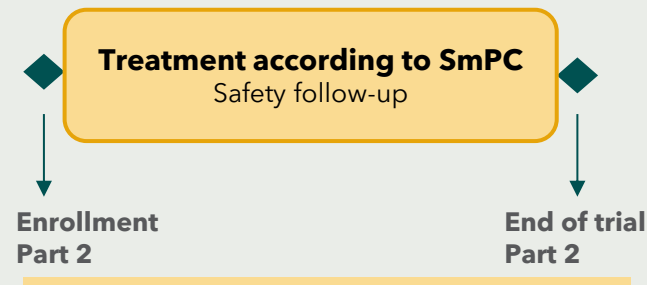
Marks a critical clinical proof-of-concept for PDCs to be developed for patients with CNS tumors, establishing a path for the PDCs beyond hematology with the opportunity to support patients and address a global glioblastoma market, estimated to grow into an 8 billion USD market by 2035.

OP-701 (INSULA) study design and status

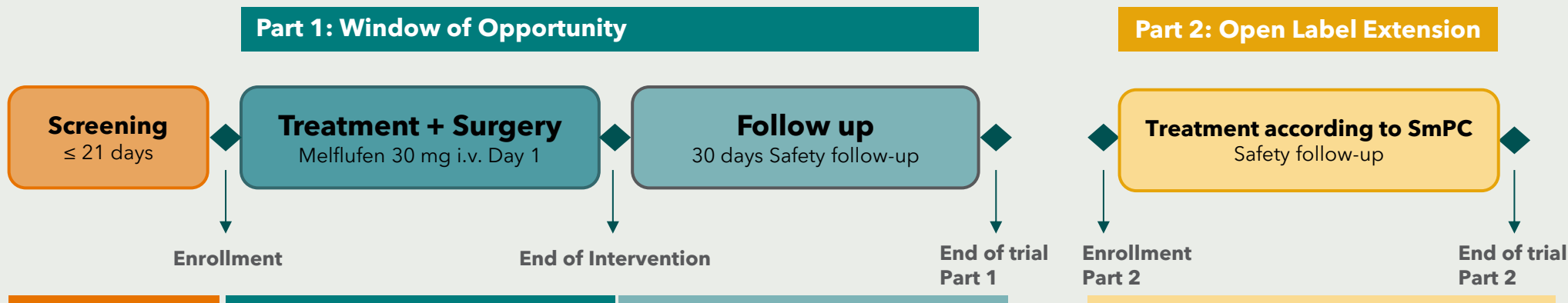
Part 1: Window of Opportunity



Part 2: Open Label Extension



OP-701 (INSULA) study design and status



Study Design & Site:

A Window-of-Opportunity (WoO) study with an optional Open-label extension study part conducted at Oslo University Hospital, enrolling patients with recurrent glioblastoma scheduled for surgery.

Part 1: Window of Opportunity

- Target enrollment: Approximately 10 patients.
- Patient enrollment: 3 patients received study medication to date
- Evaluated patients: 3 patients surgically evaluated with confirmed blood-brain barrier penetration and intra-tumoral PK data in line with pre-clinical models.

Open-Label Extension (OLE):

- Evaluated surgical patients transitioning into continuous post-operative treatment.
- Patient 1: 5 monthly cycles/doses completed to date; well tolerated with no new tumor-related neurological symptoms.
- Patient 2: Transitioned into OLE 2 cycles to date.

Next steps and future milestones



Complete study recruitment of cohort 1 and open cohort 2

Finalize enrolment to reach the planned 5 patients in cohort 1 and open cohort 2 to recruit the full study of approximately 10 glioblastoma patients at Oslo University Hospital.



Full data readout

Complete pharmacokinetic (PK) and pharmacodynamic analyses to gain further scientific insights that can inform next steps.



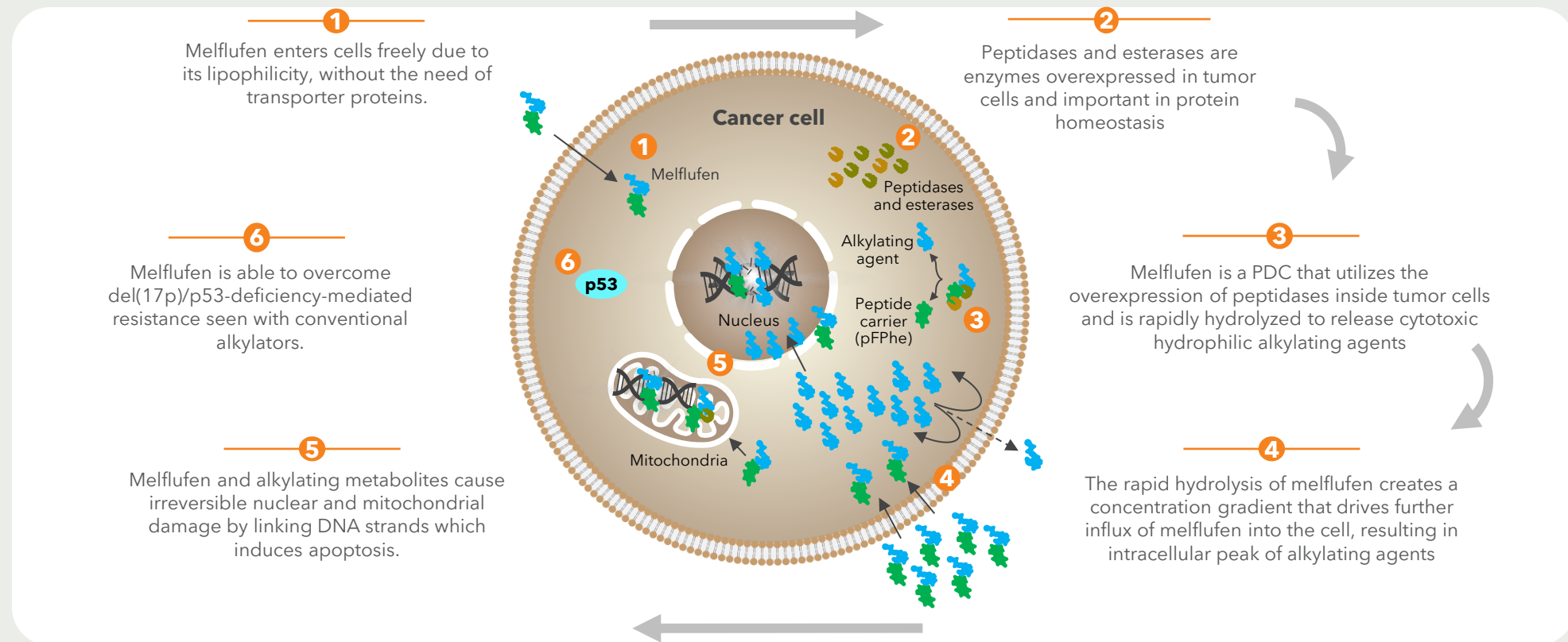
Advance the PDC pipeline

Utilize the human mechanistic proof-of-concept to inform the regulatory pathway and clinical development for PDCs in neuro-oncology.



Our pipeline opportunity

PDCs enhance alkylation through enzymatic enrichment and dual targeting of both nuclear and mitochondrial DNA



Del, deletion; PDC, peptide-drug conjugate; pFPhe, p-L fluoro-phenylalanine ethyl ester; melflufen, melphalan flufenamide; p53, tumor suppressor protein 53.

1. Chauhan D, et al. *Clin Cancer Res*. 2013;19(11):3019-31.
2. Wickström M, et al. *Oncotarget*. 2017;8(39):66641-66655.
3. Kumari R, et al. *Br J Cancer*. 2021;124(8):1428-1436.
4. Miettinen JJ, et al. *Cancers (Basel)*. 2021;13(7):1527.
5. Westermarck U, et al. *Biochem Biophys Res Commun*. 2023;656:122-130.
6. Ray A, et al. *Br J Haematol*. 2016;174(3):397-409.
7. Mateos MV, et al. ASH 2020. Poster 3237.

Our PDC Platform:

validated science, **limitless** potential

Validated scientific breakthrough

Our proprietary technology is a "smart" system designed to bypass healthy tissue and concentrate cancer-killing power directly inside cancer cells

Overcoming treatment resistance

Our platform is engineered to overcome common resistance pathways (such as p53 mutations) that often stop traditional drugs from working

Real-world proof-of-concept

With over SEK 31 million in Q2-26 sales and 1,000+ patients treated since launch, Pepaxti has proven that this science delivers results in clinical practice

Growth beyond myeloma

We are deploying this same validated mechanism to target multi-billion dollar markets like Glioblastoma, evolving from a niche player into a pioneer in difficult-to-treat cancers

Glioblastoma

A brain tumor with high unmet need and growing market potential

Most aggressive brain cancer – grows fast, invariably relapses, and has no cure.

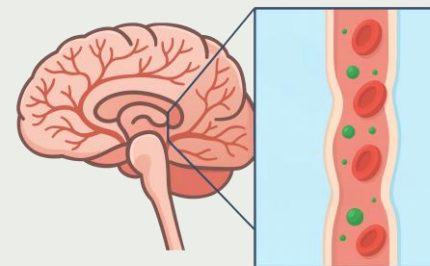
Rare but severe – affects about 3-4 people per 100,000 each year, usually above the age of 60.

Poor survival – even with treatment, patients live only about 12-15 months after diagnosis.

New, brain-penetrating therapies are urgently needed.



Glioblastoma



Blood brain barrier

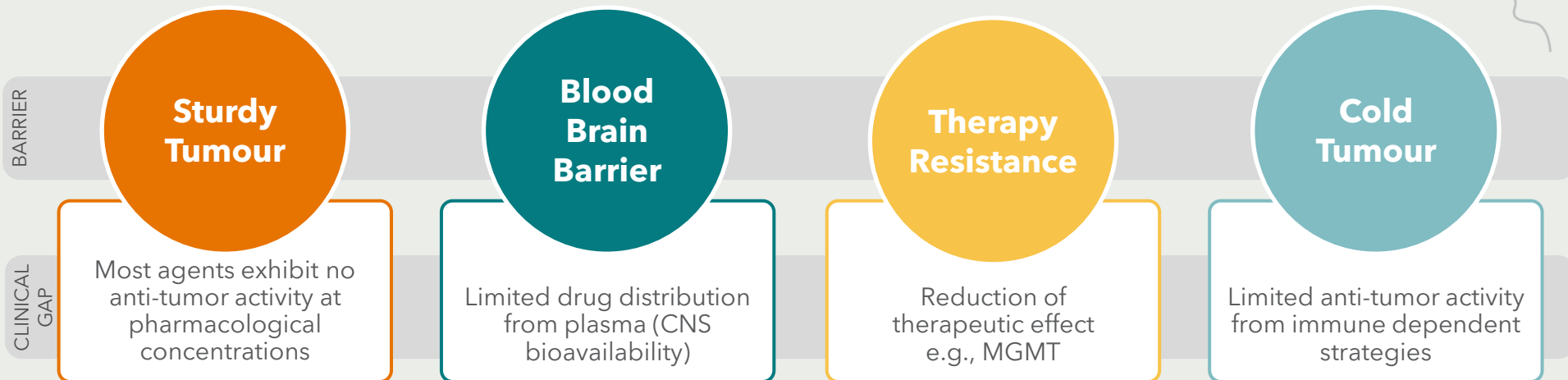


The barrier

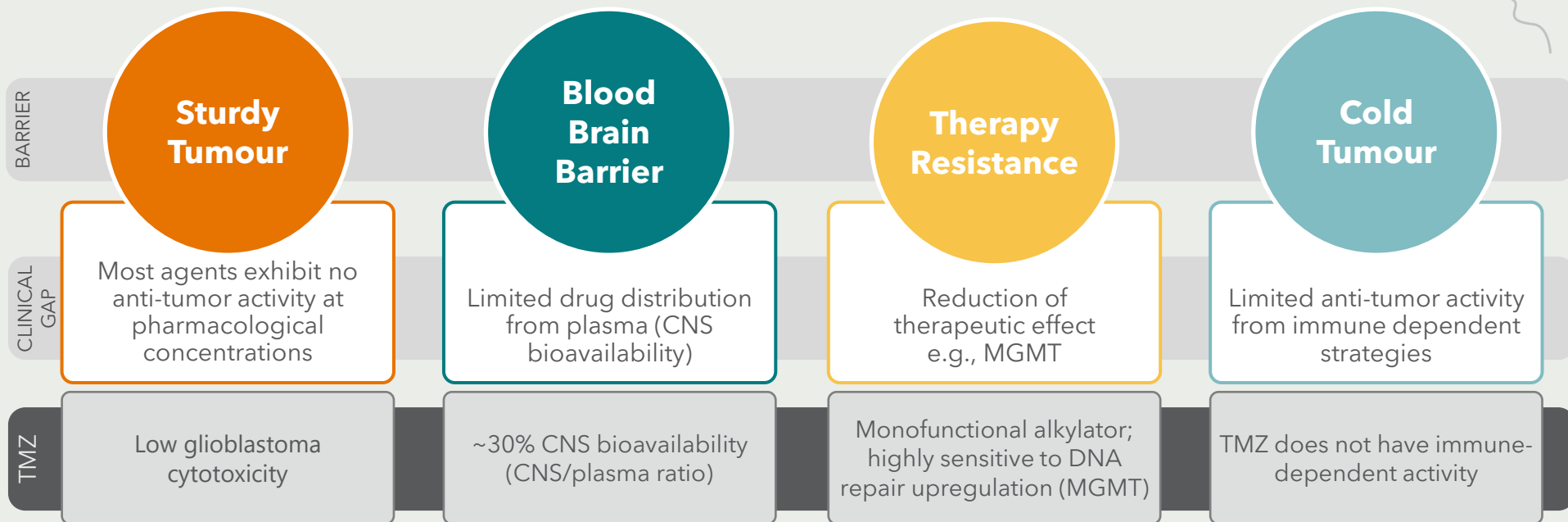
The challenge



No major therapeutic breakthrough since TMZ approval in 2005



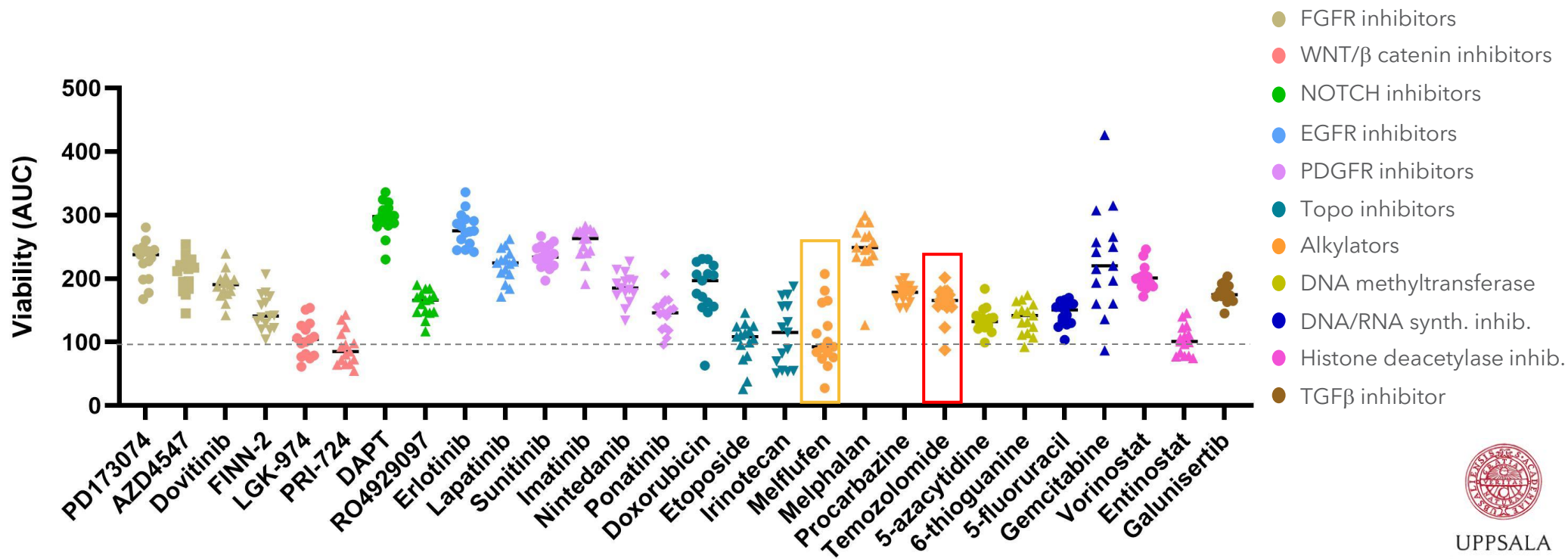
Current standard of care: temozolomide (TMZ)





Our answer

Viability in 29 treated glioblastoma cell cultures



UPPSALA
UNIVERSITET



Melflufen is one of the **most efficacious drugs** on glioblastoma in this drug screen. **Clearly superior** to the standard of care: temozolomide.

Potential of PDCs

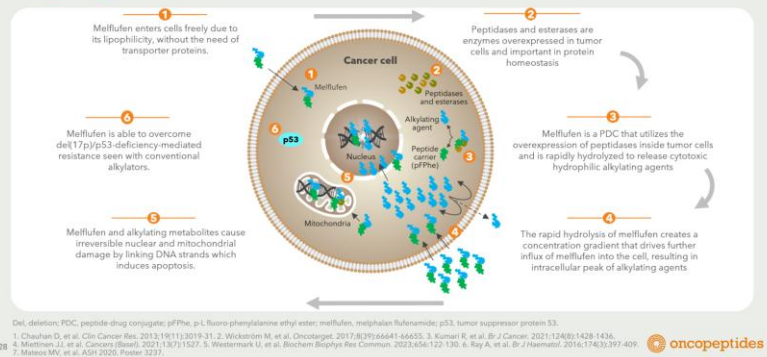
Critical BBB passage confirmed

- OPD5 has shown promising preclinical data with good activity at pharmacological concentrations
- OPD5 has shown an efficient blood-brain barrier penetration and strong tumor reduction in preclinical models

Conclusion

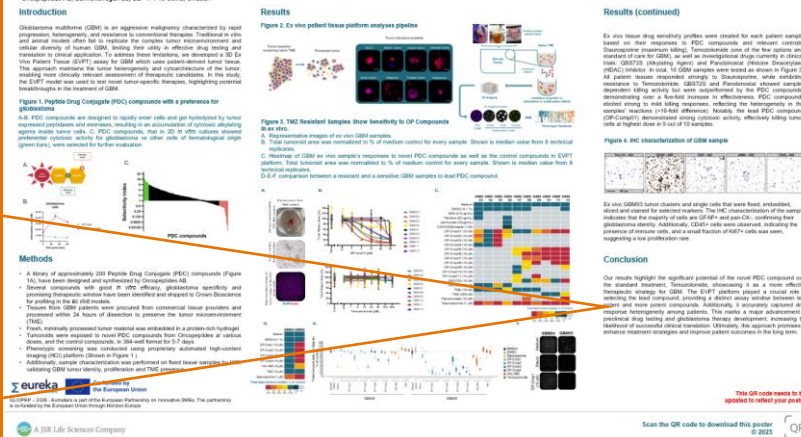
Our results highlight the significant potential of the novel PDC compound over the standard treatment, Temozolomide, showcasing it as a more effective therapeutic strategy for GBM. The EVPT platform played a crucial role in selecting the lead compound, providing a distinct assay window between less potent and more potent compounds. Additionally, it accurately captured drug response heterogeneity among patients. This marks a major advancement in preclinical drug testing and glioblastoma therapy development, increasing the likelihood of successful clinical translation. Ultimately, this approach promises to enhance treatment strategies and improve patient outcomes in the long term.

PDCs enhance alkylation through enzymatic enrichment and dual targeting of both nuclear and mitochondrial DNA



A Preclinical Ex Vivo Model for Glioblastoma Captures Patient Heterogeneity in Drug Response

Ezgi Kaya Akbay¹, Ulrica Westmark¹, Kristin Hammer¹, Luca Gaudini¹, Jelle Flash¹, Emma Spangaard¹, Stefan Svensson Gelius¹, Marit Pulver¹ and Natalia Betsina¹
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Expanding beyond blood cancer: Oncopeptides at EANO 2026

What is EANO?

- **Europe's leading brain tumor congress**

The European Association of Neuro-Oncology, the premier European medical forum dedicated entirely to brain tumors.

- **Why it matters**

Gathers leading global neuro-oncologists, researchers, and industry peers and delivers high-level international visibility and third-party scientific validation.

Scientific recognition at EANO validates that Oncopeptides' technology platform has multi-disease potential—expanding beyond blood cancer into hard-to-treat solid brain tumors.

Oncopeptides' attendance

- **First major entry into brain cancer**

Presenting 3 studies applying our PDC technology to glioblastoma, a cancer with few treatment options.

- **Reaching the brain**

Confirms our drugs cross the protective blood-brain barrier to hit resistant tumor cells.

- **Prestigious award nomination**

One poster was shortlisted for the EANO 2026 Poster Award, validating our research quality.

- **Clinical pipeline expansion**

Supports our ongoing World-of-Opportunity clinical study, proving platform value beyond multiple myeloma.

Mitochondrial function critical



Review

Targeting Mitochondria in Glioma: New Hopes for a Cure

Lidia Gatto ^{1,*}, Vincenzo Di Nunno ¹, Anna Ghelardini ², Alicia Tosoni ¹, Stefania Bartolini ¹, Sofia Asoli ^{3,4}, Stefano Ratti ⁵, Anna Luisa Di Stefano ^{6,7} and Enrico Franceschi ¹

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The promise of mitochondria in the treatment of glioblastoma: a brief review

Review | [Open access](#) | Published: 09 February 2025

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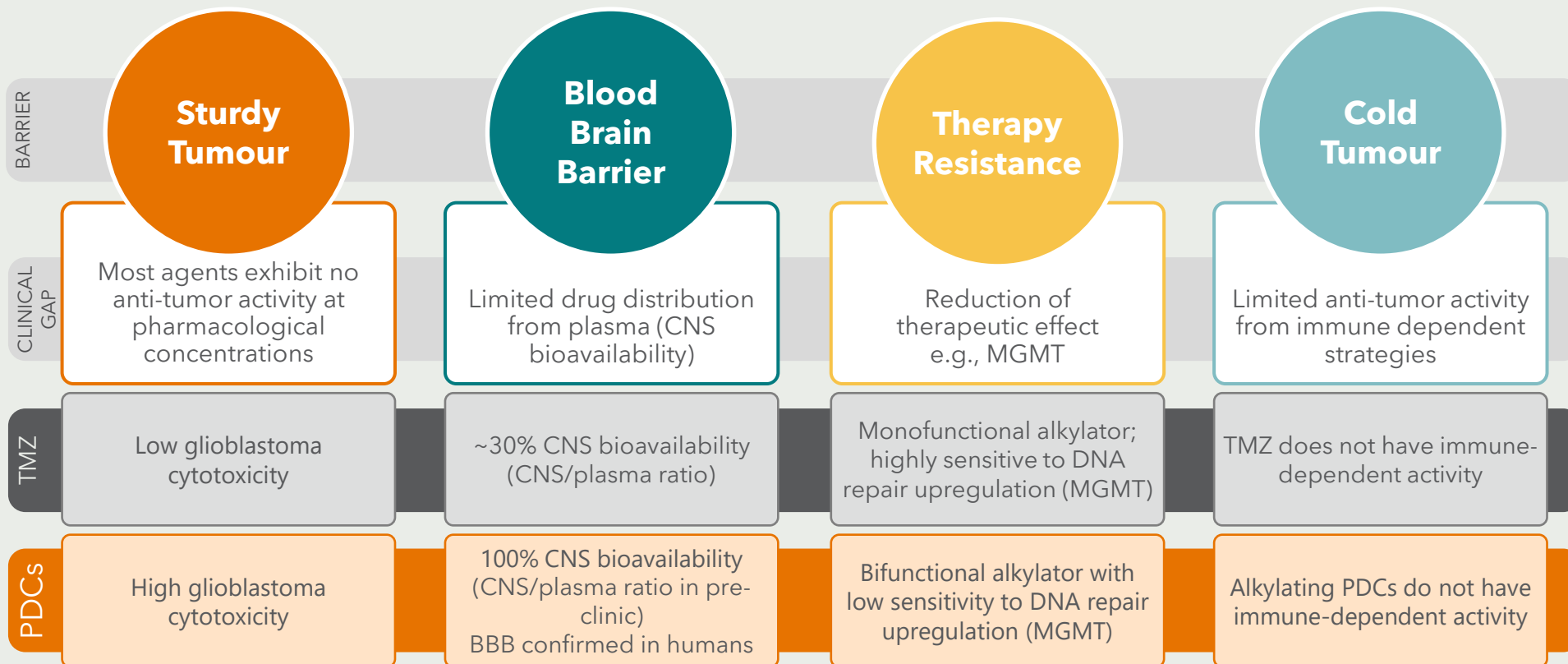
Check for updates

Functional mitochondrial respiration is essential for glioblastoma tumour growth

Petra Brisudova ^{1,2}, Dana Stojanovic ^{1,2}, Jaromir Novak ^{1,2}, Zuzana Nahacka ¹, Gabriela Lopes Oliveira ^{1,3,4,5}, Ondrej Vanatko ^{6,7}, Sarka Dvorakova ¹, Berwini Endaya ¹, Jaroslav Truksa ¹, Monika Kubiskova ^{6,7}, Alice Foltynova ^{6,7}, Daniel Jirak ⁸, Natalia Jirat-Ziolkowska ^{8,9}, Lukas Kucera ¹⁰, Karel Chalupsky ¹⁰, Krystof Klima ¹⁰, Jan Prochazka ¹⁰, Radislav Sedlacek ¹⁰, Francesco Mengarelli ¹¹, Patrick Orlando ¹¹, Luca Tiano ¹¹, Paulo J. Oliveira ^{3,4}, Carole Grasso ¹², Michael V. Berridge ¹², Renata Zabalova ¹³, Miroslava Anderova ^{6,13} and Jiri Neuzil ^{1,2,13,14}

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Alkylating PDCs (**Pepaxti, OPD5, OPDC3**)



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Q&A

Bringing hope through science

