



## Panbela Announces Publication of Clinical Data Titled:

### *Phase 1 study of high-dose DFMO, celecoxib, cyclophosphamide and topotecan for patients with relapsed neuroblastoma: A New Approaches to Neuroblastoma Therapy Trial*

- **A Phase 1 clinical study showed that high dose DFMO treatment in combination with chemotherapy may provide therapeutic benefits to heavily pretreated patients with neuroblastoma.**

**MINNEAPOLIS, January 18, 2024 (GLOBE NEWSWIRE)-- Panbela Therapeutics, Inc.** (Nasdaq: PBLA), a clinical stage biopharmaceutical company developing disruptive therapeutics for the treatment of patients with urgent unmet medical needs announces the publication of clinical data from studies of CPP-1X (also known as  $\alpha$ -Difluoromethylornithine (DFMO) or Eflornithine) in neuroblastoma. According to Hogarty et al, children with relapsed refractory neuroblastoma have dismal outcomes and new therapeutic options are needed. Data published in the British Journal of Cancer investigated the tolerability and activity of depleting polyamines by high dose CPP-1X and celecoxib in combination with standard of care chemotherapy in heavily pretreated neuroblastoma patients. Results showed that DFMO treatment was well tolerated, and the median time-to-progression was 19.8 months. The work reflects the Company's previous collaboration with New Advances in Neuroblastoma Therapy Consortium (NANT) (<https://www.nant.org/>). A link to the publication can be found here: <https://www.nature.com/articles/s41416-023-02525-2>.

From the Phase 1 dose range finding study of CPP-1X in heavily pretreated neuroblastoma patients, CPP-1X was well tolerated. The best overall response included 2 partial responses (PR), 4 minor responses (MR), 10 Stable disease (SD), 7 progressive disease (PD) and 1 unevaluable. All patients with an overall response of PR or MR sustained this response until stopping or completing protocol therapy. The overall objective response rate (CR+PR) was 9% and rate of any response (CR+PR+MR) was 26%. At 2 years, PFS (progression free survival) for the entire cohort was 29.5%. Notably, three patients completed protocol therapy and remain without disease progression or event at >4 years from treatment end in the absence of additional therapy.

These results build upon the recent FDA approval of CPP-1X or DFMO to reduce the risk of relapse in adult and pediatric patients with high-risk neuroblastoma (HRNB) who have demonstrated at least a partial response to prior multiagent, multimodality therapy including anti-GD2 immunotherapy. Results from these studies suggest that CPP-1X is a safe, oral treatment option that may improve response rates in heavily pretreated relapsed refractory neuroblastoma patients and are the basis for the ongoing ANBL-1821 Phase 2 trial.

"We are excited about the publication of these Phase 1 trial results in light of the recent DFMO FDA approval for patients in maintenance therapy. From this dose escalation study, our collaborators were able to demonstrate high dose DFMO is well tolerated and demonstrated activity in patients with heavily pretreated neuroblastoma," said Elizabeth Bruckheimer, PhD, Vice President & Chief Scientific Officer of Panbela. "Moreover, three patients remain alive over four years from treatment end without any additional therapy which suggests that high dose DFMO treatment in combination with chemotherapy may be a potential treatment option for this high unmet need population."

"Overall, these results in addition to the recent approval of DFMO as a maintenance therapy, suggests a role for polyamine inhibition therapy for neuroblastoma that may impact other cancer types such as prostate cancer. We are excited by these results and the potential role for CPP-1X in the clinical management of neuroblastoma and cancer as a whole." said Dr. Bruckheimer. "These studies were the basis for the ongoing Children's Oncology Group Phase II trial in relapsed refractory neuroblastoma to support the goal of developing effective novel therapies for patients with unmet medical needs."

First author Michael Hogarty, MD, Professor of Pediatrics at the University of Pennsylvania, and Children's Hospital of Philadelphia said, "The results from the Phase 1 study have built upon the preclinical work performed in my laboratory demonstrating a role of deep polyamine depletion as a potential therapeutic target for relapsed or refractory neuroblastoma. By understanding the underlying biology and role of MYC signaling and the polyamine pathway in neuroblastoma, we are able to show the potential impact of high-dose DFMO in neuroblastoma."

### **About Panbela**

Panbela Therapeutics, Inc. is a clinical-stage biopharmaceutical company developing disruptive therapeutics for patients with urgent unmet medical needs. Panbela's lead assets are Ivospemini (SBP-101) and Flynpovi. Further information can be found at [www.panbela.com](http://www.panbela.com). Panbela's common stock is listed on The Nasdaq Stock Market LLC under the symbol "PBLA".

### **Cautionary Statement Regarding Forward-Looking Statements**

*This press release contains "forward-looking statements," including within the meaning of the Private Securities Litigation Reform Act of 1995. Forward-looking statements can be identified by words such as: "anticipate," "believe," "can," "design," "expect," "focus," "intend," "may," "plan," "positioned," "potential," and "will." All statements other than statements of historical fact are statements that should be deemed forward-looking statements. Forward-looking statements are neither historical facts nor assurances of future performance. Instead, they are based only on our current beliefs, expectations, and assumptions regarding the future of our business, future plans and strategies, projections, anticipated events and trends, the economy and other future conditions. Because forward-looking statements relate to the future, they are subject to inherent uncertainties, risks and changes in circumstances that are difficult to predict and many of which are outside of our control. Our actual results and financial condition may differ materially and adversely from the forward-looking statements. Therefore, you should not rely on any of these forward-looking statements. Important factors that could cause our actual results and financial condition to differ materially from those indicated in the forward-looking*

*statements include, among others, the following: (i) our ability to obtain additional funding to execute our business and clinical development plans; (ii) progress and success of our clinical development program; (iii) the impact of the current COVID-19 pandemic on our ability to conduct our clinical trials; (iv) our ability to demonstrate the safety and effectiveness of our product candidates: ivospemin (SBP-101) and eflornithine (CPP-1X); (v) our reliance on a third party for the execution of the registration trial for our product candidate Flynpovi ; (vi) our ability to obtain regulatory approvals for our product candidates, SBP-101 and CPP-1X in the United States, the European Union or other international markets; (vii) the market acceptance and level of future sales of our product candidates, SBP-101 and CPP-1X; (viii) the cost and delays in product development that may result from changes in regulatory oversight applicable to our product candidates, SBP-101 and CPP-1X; (ix) the rate of progress in establishing reimbursement arrangements with third-party payors; (x) the effect of competing technological and market developments; (xi) the costs involved in filing and prosecuting patent applications and enforcing or defending patent claims; (xii) our ability to maintain the listing of our common stock on a national securities exchange; and (xiii) such other factors as discussed in Part I, Item 1A under the caption "Risk Factors" in our most recent Annual Report on Form 10-K, any additional risks presented in our Quarterly Reports on Form 10-Q and our Current Reports on Form 8-K. Any forward-looking statement made by us in this press release is based on information currently available to us and speaks only as of the date on which it is made. We undertake no obligation to publicly update any forward-looking statement or reasons why actual results would differ from those anticipated in any such forward-looking statement, whether written or oral, whether as a result of new information, future developments or otherwise.*

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