U.S. FOOD & DRUG ADMINISTRATION

Office of Orphan Products Development
Food and Drug Administration
WO32- 5295
10903 New Hampshire Avenue
Silver Spring, MD 20993

Veristat LLC
134 Turnpike Road, Suite 200
Southborough, Massachusetts 01772

Re: Designation request # DRU-2025-10725
Dated: 2/3/2025
Received: 2/4/2025

This letter responds to your request submitted on behalf of Gate2Brain S.L. for orphan-drug designation of the active metabolite of irinotecan, SN-38, in its lactone form (SN-38L) for "treatment of glioma."

Pursuant to section 526 of the Federal Food, Drug, and Cosmetic Act (21 U.S.C. 360bb), your orphan-drug designation request of 7-ethyl-10-hydroxycamptothecin is granted for *treatment of malignant glioma.* Please be advised that it is the active moiety or principal molecular structural features of the drug[1] and not the formulation of the drug that is designated.

If your drug receives marketing approval for an indication broader than what is designated, it may not be entitled to exclusive marketing rights under section 527 (21 U.S.C.360cc). Therefore, prior to submission of your marketing application, we request that you compare the drug's orphan designation with the proposed marketing indication and submit additional information to amend the orphan-drug designation if warranted. 21 CFR 316.26.

If an otherwise same drug is approved for the same indication before you obtain marketing approval of your drug, you will have to demonstrate that your drug is clinically superior to the already approved otherwise same drug in order to be eligible for orphan-drug exclusivity. Failure to demonstrate clinical superiority over the already approved otherwise

[1] The term "drug" in this letter includes drug and biological products.

same drug will result in your drug not being eligible for orphan-drug exclusivity. 21 CFR 316.34(c).

You must submit to the Office of Orphan Products Development a brief progress report of drug development within 14 months after the date of this letter and annually thereafter until marketing approval. 21 CFR 316.30. Please submit the annual report via email to orphan@fda.hhs.gov.

Please notify this office within 30 days of submitting a marketing application for the drug's designated use. Once your marketing application is approved, please contact our office at orphan@fda.hhs.gov to assess eligibility for orphan-drug exclusivity.

It is your responsibility to update the Office of Orphan Products Development (OOPD) if the contact information for the orphan-drug designation changes. Submitting changes regarding the contact information to the Center for Drug Evaluation and Research (CDER) or Center for Biologics Evaluation and Research (CBER) is not sufficient to update the orphan-drug designation file in OOPD. The sponsor should submit any changes regarding the contact information for the orphan-drug designation described in 21 CFR 316.20(b)(2) to the orphan-drug designation file in OOPD.

Under 21 CFR 316.27(a), a sponsor may transfer ownership of (or any beneficial interest in) an orphan-drug designation of a drug to a new sponsor. At the time of the transfer, the new and former owners are required to submit specific documentation as described in 21 CFR 316.27(a). Such documentation should be submitted directly to the orphan-drug designation file in OOPD. Please note that transferring ownership of an Investigational New Drug (IND) application, New Drug Application (NDA), or Biologics License Application (BLA) does not change the ownership of the orphan-drug designation. Therefore, even if documentation is submitted to CDER or CBER to change ownership of an IND, NDA, or BLA, if the new and former owners intend to transfer ownership of the orphan-drug designation, they should also submit the documentation described in 21 CFR 316.27(a) to OOPD directly.

Should you have any questions, please contact our office by phone at 301-796-8660 or by email at orphan@fda.hhs.gov.

Sincerely,

{See appended electronic signature page}

Director
Office of Orphan Products Development

FDA U.S. FOOD & DRUG ADMINISTRATION

Devanand Jillapalli

Digitally signed by Devanand Jillapalli
Signed on behalf of Sandra Retzky
Date: 4/16/2025 5:04 PM EDT
GUID: 91970