

Fast Track Development Program, August 27, 2010 - Corifact

Designation and Review Programs SOPP 8414 Appendix 7

Review for Determining Fast Track Development Program Designation

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Receipt Date of Fast Track Request: 08/18/2010 (?) Date of Review Memo: August 27, 2010

BLA number: 125385

Amendment number: 0

Product: Factor XIII Concentrate (Human)

Sponsor: CSL Behring

Condition for which the drug is intended and the specific anticipated benefits of use: The proposed indication for Factor XIII Concentrate is routine prophylactic treatment of congenital Factor XIII deficiency.

A. Consideration of Fast Track Elements:

1. Is the aspect(s) of the condition anticipated to be benefited serious or life-threatening? Yes No
2. Does the drug show potential (given its stage of development) to treat this serious aspect of the condition? Yes No
3. Is the drug development program designed to determine whether the drug will affect a serious aspect of the condition? (Degree of specificity should be appropriate to the stage of development) Yes No
4. Is there any accepted/approved treatment for the same serious or life-threatening aspect of the condition being studied? Yes No

Recommendation: The Division of Hematology is granting the fast track designation under **21 CFR 601, Subpart E, §601.41** for the submitted clinical development.

The Factor XIII Concentrate is intended to treat a **serious and life-threatening condition**; Factor XIII deficiency is a rare hereditary bleeding disorder of high life-threatening potential. Although cryoprecipitate and fresh frozen plasma provide a source of factor XIII, these products carry risks of blood-borne disease, viral transmission, fluid overload, immunologic reactions, etc. There are only two commercial Factor XIII products in Europe, which are not approved in the US. Fibrogammin-P (CSL Behring UK Limited) is available in the United States only under IND/clinical ----- (b)(4)-----, or for prophylactic intervention. There is currently no approved factor XIII replacement therapy in the US. Therefore the product is addressing an **unmet medical need**.

FDA may grant marketing approval for this biological product based on the submitted adequate and well-controlled trial establishing the effect of Human Plasma-derived Factor XIII concentrate on a surrogate endpoint (Factor XIII through activity levels).

The Sponsor was notified that the approval under this section will be also subject to the post-marketing requirement safety and efficacy study to prove the relation of the surrogate endpoint to clinical benefit. This study is ongoing (under BB-IND (b)(4)).

Fast Track Development Program is (check one)

granted _____

denied _____

Reviewer Signature/Date: Daniela J. Vanco, M.D./August 27, 2010
Concurrence by Branch Chief:

Signature/Date _____

Concurrence by Division Director:

Signature/Date _____