

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? X Yes No
2. Does the drug show potential (given its stage of development) to treat this serious aspect of the condition? X 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) X Yes No
4. Is there any accepted/approved treatment for the same serious or life-threatening aspect of the condition being studied? Yes X 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____X_____

denied_____

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

Signature/Date_____

Concurrence by Division Director:

Signature/Date_____