



4164-01-P

DEPARTMENT OF HEALTH AND HUMAN SERVICES

Food and Drug Administration

[Docket No. FDA-2014-N-0229]

Issuance of Priority Review Voucher; Rare Pediatric Disease Product

AGENCY: Food and Drug Administration, HHS.

ACTION: Notice.

SUMMARY: The Food and Drug Administration (FDA) is announcing the issuance of a priority review voucher to the sponsor of a rare pediatric disease product application. The Federal Food, Drug, and Cosmetic Act (the FD&C Act), as amended by the Food and Drug Administration Safety and Innovation Act (FDASIA), authorizes FDA to award priority review vouchers to sponsors of rare pediatric disease product applications that meet certain criteria. FDA has determined that CHOLBAM (cholic acid), manufactured by Asklepiion Pharmaceuticals, LLC, meets the criteria for a priority review voucher.

FOR FURTHER INFORMATION CONTACT: Larry Bauer, Rare Diseases Program, Center for Drug Evaluation and Research, Food and Drug Administration, 10903 New Hampshire Ave., Silver Spring, MD 20993-0002, 301-796-4842, FAX: 301-796-9858, email:

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SUPPLEMENTARY INFORMATION: FDA is announcing the issuance of a priority review voucher to the sponsor of a rare pediatric disease product application. Under section 529 of the FD&C Act (21 U.S.C. 360ff), added by FDASIA, FDA will award priority review vouchers to

sponsors of rare pediatric disease product applications that meet certain criteria. FDA has determined that CHOLBAM (cholic acid), manufactured by Asklepiion Pharmaceuticals, LLC, meets the criteria for a priority review voucher. CHOLBAM (cholic acid) is a bile acid indicated for the treatment of bile acid synthesis disorders due to single enzyme defects and as adjunctive treatment of peroxisomal disorders, including Zellweger spectrum disorders in patients who exhibit manifestations of liver disease or steatorrhea or complications from decreased fat soluble vitamin absorption. Bile acid synthesis disorders is a group of rare congenital disorders caused by the absence or malfunction of an enzyme involved in an important metabolic pathway, leading to a failure to produce normal bile acids.

For further information about the Rare Pediatric Disease Priority Review Voucher Program and for a link to the full text of section 529 of the FD&C Act, go to <http://www.fda.gov/ForIndustry/DevelopingProductsforRareDiseasesConditions/RarePediatricDiseasePriorityVoucherProgram/default.htm>.

For further information about CHOLBAM (cholic acid), go to the Drugs@FDA Web site at <http://www.accessdata.fda.gov/scripts/cder/drugsatfda/index.cfm>.

Dated: April 2, 2015.

Leslie Kux,

Associate Commissioner for Policy.

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