

FOOD AND DRUG ADMINISTRATION (FDA)
Center for Drug Evaluation and Research (CDER)
Endocrinologic and Metabolic Drugs Advisory Committee (EMDAC) Meeting

FDA White Oak Campus, Building 31, the Great Room,
White Oak Conference Center (Rm. 1503), Silver Spring, MD
November 19, 2013

AGENDA

The committee will discuss Biologics License Application (BLA) 125460 for VIMIZIM (elosulfase alfa), manufactured by BioMarin Pharmaceutical, Inc., for the treatment of Mucopolysaccharidosis Type IVA (Morquio A syndrome). Morquio A syndrome is a rare congenital disorder caused by the absence or malfunctioning of an enzyme involved in an important metabolic pathway, leading to problems with bone development, growth and movement.

8:00 a.m.	Call to Order and Introduction of Committee	Robert J. Smith, MD Acting Chairperson, EMDAC
8:10 a.m.	Conflict of Interest Statement	Cindy Hong, PharmD Acting Designated Federal Officer, EMDAC
8:20 a.m.	FDA Introductory Remarks	Andrew E. Mulberg, MD Division Deputy Director Division of Gastroenterology and Inborn Errors Products (DGIEP) Office of Drug Evaluation (ODE-III) Office of New Drugs (OND), CDER, FDA
8:30 a.m.	<u>SPONSOR PRESENTATIONS</u> Introduction	<u>BioMarin Pharmaceutical, Inc.</u> Henry Fuchs, MD Chief Medical Officer BioMarin Pharmaceutical Inc.
	Description of Mucopolysaccharidosis Type IVA (MPS IVA)	Christian Hendriksz, MB ChB, MSc, MRCP, FRCPCH Birmingham Children's Hospital NHS Foundation Trust, United Kingdom Clinical Director – Acute Medicine, Salford Royal NHS Foundation Trust
	Clinical Trial Results	Christine Haller, MD Senior Group Medical Director BioMarin Pharmaceutical Inc.

FOOD AND DRUG ADMINISTRATION (FDA)
Center for Drug Evaluation and Research (CDER)
Endocrinologic and Metabolic Drugs Advisory Committee (EMDAC) Meeting

November 19, 2013

Agenda (cont.)

SPONSOR PRESENTATIONS (CONT.)

Risk:Benefit

**Christian Hendriksz, MB ChB, MSc,
MRCP, FRCPCH**

Conclusion

Henry Fuchs, MD

9:45 a.m. Clarifying Questions from the Committee

10:25 a.m. **BREAK**

10:40 a.m. **FDA PRESENTATIONS**

Evaluating Treatment Benefit in MPS IVA

Jessica J. Lee, MD
Medical Team Leader
DGIEP, ODE-III, OND, CDER, FDA

FDA Review of Clinical Efficacy and Safety

Tamara Johnson, MD
Medical Officer
DGIEP, ODE-III, OND, CDER, FDA

11:30 a.m. Clarifying Questions from the Committee

12:00 p.m. **LUNCH**

1:00 p.m. Open Public Hearing

2:00 p.m. Questions to the Committee/Committee Discussion

3:00 p.m. **BREAK**

3:15 p.m. Questions to the Committee/Committee Discussion

5:00 p.m. **ADJOURNMENT**