



**National Organization for Rare Disorders
Remarks before the
FDA Arthritis Advisory Committee
Silver Spring, Maryland
February 9, 2016**

Good afternoon. My name is Paul Melmeyer, Associate Director of Public Policy at the National Organization for Rare Disorders (NORD). I have no disclosures.

I am here today on behalf of the men, women and children in the United States suffering with one of the 7,000 known rare diseases that, in the aggregate affect well over 30 million people.

NORD, a 501(c)(3) organization, is a unique federation of voluntary health organizations dedicated to helping people with rare "orphan" diseases and assisting the organizations that serve them. NORD's mission is to ensure that all people with rare diseases have access to diagnostics and therapies that extend and improve their lives, and that the United States maintain a regulatory environment that encourages the development and timely approval of safe and effective diagnostics and treatments for patients affected by rare diseases.

Biologics represent the future of rare disease treatments. Biologics treat rare and chronic diseases in an innovative and rejuvenating manner that small molecule treatments are unable to do.

NORD is a proud member of the Patients for Biologics Safety and Access Coalition, and we would like to reiterate many of their established positions. We are concerned that the Agency has not yet issued final guidance on various biosimilar policies that impact patient safety, such as interchangeability, naming, and labeling. NORD also supports the institution of unique and non-proprietary naming to eliminate confusion among patients and prescribers.

We support the complete labeling of Biosimilars to identify the product as a biosimilar, and indicate if it is interchangeable with the reference product. We encourage the FDA to provide greater educational services to rare disease patients and their physicians to better understand the unique nuances of Biosimilars.

Outside of our shared positions with PBSA, we are also concerned with the FDA's decision to discuss the potential determination of biosimilarity of CT-P13 in the pediatric ulcerative colitis indication. This orphan indication in the reference product holds orphan drug exclusivity until September 23, 2018. For over thirty years, NORD has fiercely defended the Orphan Drug Act and its valuable incentives for the innovative development of orphan therapies. Actions that weaken the exclusivity protections within the program are thus particularly troubling. This potential weakening of incentives for orphan development could lead to fewer products being developed for the rare disease patient community.

While we have our concerns with extrapolation, if extrapolation is to occur, then it needs to carefully and definitively preclude an extrapolation to an ODA protected indication. This very

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issue is at stake today. By putting it on the agenda for discussion, FDA has implied that there is less than a 100% commitment to honoring the ODA in these circumstances. We urge you to make clear in your comments on this question that you consider extrapolation to a protected orphan indication as unacceptable.

Thank you again for the opportunity to participate in today's hearing.

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