National Organization for Rare Disorders
Remarks before the
FDA Cardiovascular and Renal Drugs Advisory Committee
Silver Spring, Maryland
August 5, 2013

Good afternoon. My name is Diane Edquist Dorman, Vice President for Public Policy for the National Organization for Rare Disorders (NORD). I have no personal financial relationship with Otsuka.

I am here today, not on behalf of the company before you today, but on behalf of patients and their families affected by autosomal dominant polycystic kidney disease and the men, women and children in the United States suffering with one of the 7,300 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 is committed to the identification, treatment, and cure of rare disorders through programs of education, advocacy, research, and service. 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 with rare diseases.

Rare disease research and the development of orphan therapies to treat them are unique in many respects. Patient populations are generally very small and geographically dispersed across the world. There are few researchers and biopharmaceutical companies willing to take on the financial risk associated with this vital and often life-saving work.

Today there are over 400 orphan drugs and biologics that treat an estimated 300 rare diseases. Given that there are thousands more rare diseases without any specific treatment, it is easy to understand that there are many people who can only hope that one day someone will take on the significant risk to develop a therapy for their condition.

Subpart E maintains that the agency has in place procedures to expedite the development, evaluation and marketing of new therapies that are intended "to treat persons with life-threatening and severely debilitating illnesses, especially where no satisfactory alternative therapy exists." ADPKD is such a case. Subpart E goes on to say that drugs, tolvaptan being a prime example, demand flexibility and it considers it appropriate to exercise the broadest definition of flexibility when applying the standards.

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On behalf of all rare disease patients and their families, as you continue your deliberations, NORD asks only that this Advisory Committee stand by that commitment and apply a greater degree of flexibility, keeping in mind that there are few treat options for thousands of rare diseases; in the case of ADPKD, no treatment option other than palliative care. Orphan products are highly specialized for very small populations, and patients affected by ADPKD, may be willing to accept more risk and less certainty of benefit in exchange for access to a therapy treating an unmet medical need.

NORD's hope is that the kinds of information that the patients this Committee is hearing from today may contribute toward your decision-making in assessing the benefit-risk equation of a therapy treating a life-threatening unmet medical need. We know that patients at various stages of their condition are willing to take on a greater degree of risk given the quality-of-life challenges they face every day.

Thank you.

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