Since the Orphan Drug Act of 1983 was signed into law, it has resulted in more than 2,000 designations and 350 market approvals of drugs and biologicals for rare diseases considered “orphans,” that is, diseases affecting 200,000 or fewer patients in the U.S. Currently, approximately 6,000 orphan diseases affect more than 25 million people in the U.S.

Pharmaceutical and biotech firms have responded to the need to develop new medicinal products to treat orphan diseases. The Food and Drug Administration (FDA), in turn, has more than doubled the number of investigational compounds awarded orphan designation in the last five years alone. Reflecting the urgency in developing new drugs to treat orphan diseases, the FDA has increased the number of products getting priority review status during the past decade. These trends are summarized in this Tufts CSDD Impact Report, updating an earlier review reported in Tufts CSDD Impact Report 2002 May/June;4(3).