

## **The Importance of Natural History Studies for Rare Diseases**

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According to the National Institutes of Health (NIH), there are approximately 7000 rare diseases, most of which are poorly understood and only a few hundred of which have approved therapies. Unlike more prevalent diseases, rare disease drug development poses more challenges due to the lack of information about rare diseases and candidates for clinical trials. To address the orphan drug development process, natural history studies have been introduced in research to obtain a better understanding and follow the natural progression of rare diseases. Information acquired from these studies can be used to facilitate product development and the approval process. In support of this cause, the National Organization for Rare Disorders (NORD) registry platform is an application designed to empower rare disease patient organizations to collect and manage research-grade, natural history patient reported outcomes data.