PPD®

HELPING DELIVER LIFE-CHANGING THERAPIES

RARE DISEASES

RARE DISEASE AND PEDIATRIC CENTER OF EXCELLENCE

PPD's Rare Disease and Pediatric Center of Excellence is an experienced and dedicated cross-functional leadership team with clear accountabilities. The center of excellence team addresses strategic, operational, medical and scientific challenges presented by the small, widely dispersed patient populations.

Most comprehensive rare disease center of excellence in the industry

Delivers tailored thought leadership in the design and execution of trials

Provides aligned oversight of program delivery for operational teams

The center of excellence team drives all of PPD's rare disease and pediatric activities with accountabilities that span all therapeutic areas. The team provides tailored thought leadership and innovation in the design and execution of trials, as well as aligned oversight of program delivery and clear escalation paths for operational teams. In addition, the team provides strategic insights that can inform and optimize clinical and regulatory strategy development and facilitate access to a broad array of key stakeholders, including medical experts, patients and patient advocacy groups. Our team brings a wealth of experience combined with access to data, analytics and innovative technology solutions to advance and optimize the design and delivery of complex global programs in low prevalence indications.

AN OPPORTUNITY FOR REAL IMPACT

The physical and psychological toll of a rare disease on families is incalculable. Alleviating this burden requires an integrated approach to overcoming persistent obstacles that plague rare-disease drug development: insufficient evidence on the pathophysiology of rare disease, lack of consensus on relevant endpoints, inexperienced research sites, dearth of regulatory precedents that will impact a compound's approval and finding sufficient patient numbers to participate.

At PPD, we leverage our medical, operational and regulatory expertise—combined with our real-world experience—to design and operationalize studies with a customized approach for each respective disease. Additionally, we continue to invest in next-generation capabilities to enhance the investigator, patient and caregiver experience.

FOCUSED RARE DISEASE APPROACH

Specialized workforce and operating model

- Experienced rare disease team members embedded across therapeutic areas, consulting and PPD® Laboratories
- Specific expertise in pediatric consent and assent, pharmacokinetics and formulation
- Site concierge serves as a single point of contact for sites
- Lab concierge manages critical and complex laboratory samples

Patient and caregiver focus

- Patient-centered research through Evidera evaluates patient, clinician, and caregiver outcomes, utilities and preferences
- Patient concierge serves as a single point of contact for patients to proactively guide them through the trial and manage logistical aspects
- Engagement with patient advocacy groups incorporates patients' voices into study designs

Comprehensive approach to patient identification and access

- Patient pathway mapping informs of the patient journey
- Multichannel patient identification to find small eligible patients
- Patient registry design and execution

Global footprint and infrastructure

- Access to sites and investigators in every region and the Pediatric Investigator Network (PIN), accelerates and optimizes the development of therapies
- Global real-world evidence solutions to optimize registration value and access
- Continuous investment in technology and data solutions

EXPERIENCE ON A GLOBAL SCALE

PPD has extensive global experience working with drug developers to design and execute successful clinical trials focused on rare disease indications across a broad spectrum of therapeutic areas.

- Cardiovascular
- Musculoskeletal
- Gastrointestinal
- Dermatology
- Immunology
- Opthalmology
- Hematology
- Neuroscience
- Oncology
- Endocrinology/Metabolic
- Respiratory

In the past five years,
we have partnered with clients on:

460+
studies
in rare diseases

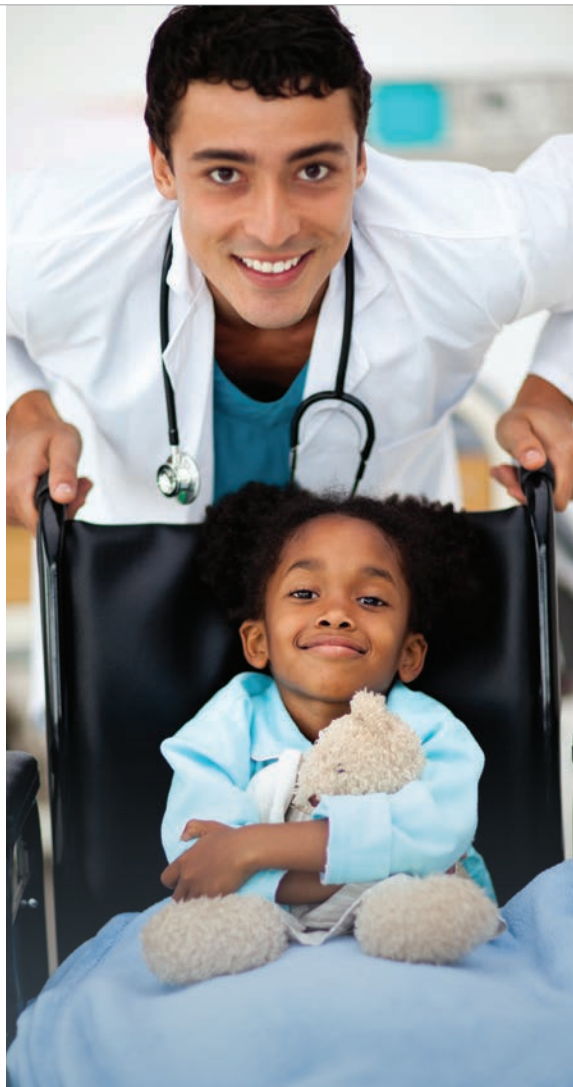
70+
countries
around the world

111,000+
patients

UNMATCHED PEDIATRIC KNOWLEDGE AND EXPERIENCE

Children comprise half of the rare disease population, and their unique needs and challenges must be considered when planning and executing clinical trials. PPD has broad experience working with pediatric populations, having executed more than 350 pediatric studies in the last five years. We provide expert guidance on:

- Consent, assent formulation and safety;
- Protocol development, feasibility, study design and study-related issues based on knowledge of current standard of care and regulatory trends in the pediatric space;
- Product development and pediatric investigational plans
- Appropriate regulatory structures and processes and access to investigators with relevant pediatric expertise.



RARE DISEASE FRAMEWORK: BUILT FOR SUCCESS

PPD deploys a four-part framework for rare disease studies that ensures an in-depth understanding of the disease, customized planning and the application of best practices in study development. This framework is executed by cross-functional, cross-therapeutic study teams that consistently work to further their knowledge of evolving science and industry advancements. The four key elements of this framework include innovative and executable study designs, rigorous feasibility and study planning, study execution and proactive pre- and post-study planning.

INNOVATIVE & EXECUTABLE STUDY DESIGNS

- Mapping of clinical care pathways
- Alternative trial designs
- Engagement of physicians and patients in study design and planning
- Endpoint and PRO development

RIGOROUS FEASIBILITY & STUDY PLANNING

- Mapping of patient journey
- Data-driven site and patient identification
- Enrollment modeling
- Site-level enrollment plans
- Comprehensive patient engagement

STUDY EXECUTION FOCUSED ON RECRUITMENT, RETENTION & QUALITY

- Standardized site training and support for naïve sites
- Travel, reimbursement and home health solutions
- High-touch site, investigator and lab engagement
- Data surveillance to ensure quality

PROACTIVE PRE- AND POST-STUDY DEVELOPMENT PLANNING

- Longitudinal site relationships
- IP access programs
- Registries, natural history studies
- HEOR and market access analysis
- Post-market safety analyses

CROSS-FUNCTIONAL, CROSS-THERAPEUTIC APPROACH

PPD®

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