PHARMALEX
CONFIDENCE BEYOND COMPLIANCE

ORPHAN MEDICINES DEVELOPMENT

A PHARMALEX SOLUTION THAT MEETS YOUR EVOLVING NEEDS

DISCOVERY / NON-CLINICAL | CLINICAL DEVELOPMENT | MARKET & LAUNCH | PRODUCT MAINTENANCE

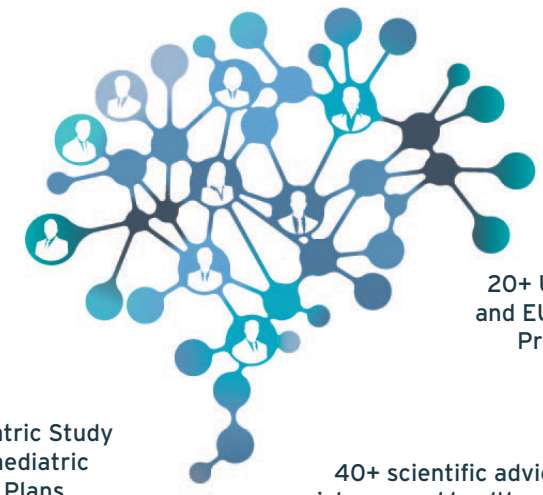
Orphan medicines are intended to treat rare diseases. Both, the EU and US drug legislation have financial and regulatory incentive systems in place to encourage developers engaging in these neglected therapeutic areas. Nevertheless, often development of a new orphan medicine follows alternative development and regulatory pathways which require specific scientific and regulatory expertise.

Our experts provide hands-on regulatory support including development consultancy, support in health authority interactions, labeling, (electronic) dossier compilation & submission, procedure management (pre- and post-approval as well as marketing authorization applications).

EXPERT KNOWLEDGE COMBINED WITH A GLOBAL FOOTPRINT:

9 out of the top 10 pharmaceutical companies are valued long-term clients

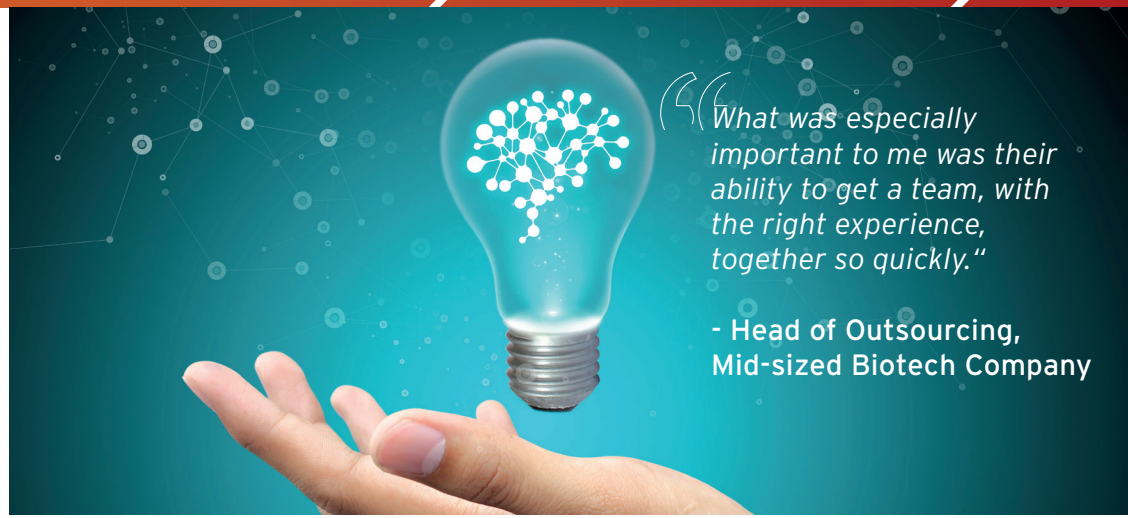
Projects with 400+MAs across 40+ countries (ICH & non-ICH regions)



20+ US Orphan Product and EU Orphan Medicinal Product designations

35+ US Pediatric Study Plans / EU Paediatric Investigation Plans

40+ scientific advices incl. assistance and health authority meetings annually with FDA, EMA and national EU agencies



"What was especially important to me was their ability to get a team, with the right experience, together so quickly."

- Head of Outsourcing, Mid-sized Biotech Company

PHARMALEX CAN OFFER SUPPORT IN THE FOLLOWING AREAS:

- Strategic advice for defining the orphan condition
- Orphan designation applications to the FDA and EMA including FDA rare pediatric disease designation (and maintenance of designations)
- Set up of integrated development plans
- Development of clinical trial designs
- Preparation and management of clinical trial applications (e.g. IND / IMPD)
- Health agency interactions (e.g. EMA protocol assistance, FDA meetings, joint EMA/FDA advice)
- Pediatric investigational plan/waiver applications to the EMA/PDCO
- Pediatric study plan/waiver applications to the FDA
- Strategic consultancy and hands-on support for EU and US expedited program eligibility applications (e.g. PRIME, Accelerated Assessment, Breakthrough Designation, Priority Review)

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DELIVERING SUCCESS WITH CONFIDENCE

PharmaLex is one of the largest providers for Development Consulting, Regulatory Affairs, Quality Management & Compliance and Pharmacovigilance, Epidemiology, and Risk Management worldwide. Our global teams of experts can take you through early strategic planning activities and non-clinical requirements to clinical development, through regulatory submission processes and finally guide you to market approval and product maintenance post-launch activities.

"Since our founding in 1994, every single one of our clients is our top priority! We lead where others follow. We help clients to go beyond compliance, to capitalize on greater efficiencies, streamline complexity and to deliver true business value.

Ask us how we can make your job easier – over 600 customers are glad they did."

Dr. Thomas Dobmeyer, CEO

PHARMALEX IN NUMBERS