PHARMALEX

ORPHAN MEDICINES DEVELOPMENT

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).

A PHARMALEX SOLUTION THAT MEETS YOUR EVOLVING NEEDS

- 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)

30+ US Orphan Product and EU Orphan Medicinal Product designations

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

50+ US Pediatric Study Plans / EU Paediatric Investigation Plans

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

InnoPHLEX

DISCOVERY / NON-CLINICAL

CLINICAL DEVELOPMENT

APPROVAL / AUTHORIZATION

PRODUCT MAINTENANCE

SourcePHLEX

DELIVERING SUCCESS WITH CONFIDENCE

PHARMALEX IS A LEADING PROVIDER OF SPECIALIZED SERVICES FOR THE PHARMA, BIOTECH AND MEDICAL DEVICE INDUSTRIES

KNOWLEDGE.
ACCELERATED.
confidence *beyond* compliance

We guide you from early strategic planning activities and non-clinical requirements through clinical development, regulatory submission processes and post-approval / maintenance post-launch activities.

Our experts use **technology-enabled solutions** to support you through the entire product lifecycle. We deliver exceptional results – going above and beyond the standard to deliver tailor-made solutions worldwide.

The PharmaLex Group now has over **1000** employees, with **33** offices in **21** countries and more than **600** satisfied clients worldwide.

Stay **one step ahead** of essentials requirements needed by health agencies. Our knowledge accelerates your business sucess...

Knowledge. Accelerated.

CONTACT US

contact@pharmalex.com

/company/pharmalexglobal

www.pharmalex.com

@PharmaLexGLOBAL

STRATEGY & CONSULTING REGULATORY AFFAIRS PHARMACOVIGILANCE QUALITY MEDICAL AFFAIRS

1000+
SUBJECT MATTER EXPERTS WORLDWIDE

25+ YEARS
OF INDUSTRY EXPERIENCE

9/10
TOP PHARMACEUTICAL COMPANIES ARE OUR SATISFIED CLIENTS

40+ NATIONALITIES
ON STAFF, INCLUDING FORMER FDA AND EMA EXPERTS

50+%
OF OUR PROJECTS ARE GLOBAL