PHARMALEX
CONFIDENCE BEYOND COMPLIANCE

CASE STUDY:
Development plan for Immune suppressant in a specific indication within the Orphan Condition tuberous sclerosis

CLIENT SIZE Medium Pharma

GEOGRAPHY EU

THERAPEUTIC AREA Oprhan product

CLIENT NEED

- Immune suppressant to be applied as subcutaneous application in a specific indication
- Client aimed for a hybrid application with reference to the originator
- Additional own studies need to add up for a new indication and administration form

PharmaLex offered support with:

- Proposing the regulatory strategy for EU and US: including Protocol assistance, PIP (partly waiver) / PSP
- Identifying bridging studies for preclinical development taking into account literature
- Propose clinical design for dose finding and pivotal study
- Identifying Pit-fall

OUR SOLUTION

PharmaLex Project Leader as single point of contact for the customer:

- Provides strategic support
- Responsible for all deliverables
- Coordinates the PharmaLex experts
- Manages timelines and costs
- Team of experts for the tasks requested.
- Leading to a regulatory strategy and an optimized proposal for additional studies to be proposed in Scientific Advices / Protocol assistance

PHARMALEX VALUE TO CLIENT

- Development plan that is recognized by the competent authorities
- Optimized resources to achieve a new orphan indication and administration form providing additional incentives and allowing a marketing advantage
- Early identification of financial risks leading to strategic decision to stop the project

DISCOVERY / NON-CLINICAL > CLINICAL DEVELOPMENT > MARKETING AUTHORIZATION / APPROVAL > PRODUCT MAINTENANCE