



Hospices Civils de Lyon

ripps



KiDS
France

DéfiScience

Maladies Rares du Développement Cérébral
et Déficience Intellectuelle

FILIÈRE NATIONALE DE SANTÉ

PRECLINICAL
PHASE

**DETERMINATION of
the TARGET of the
DRUG
(organ, receptor...)**

PHARMACOKINETICS

-

**How the ORGANISM
affects a DRUG**

TOXICITY

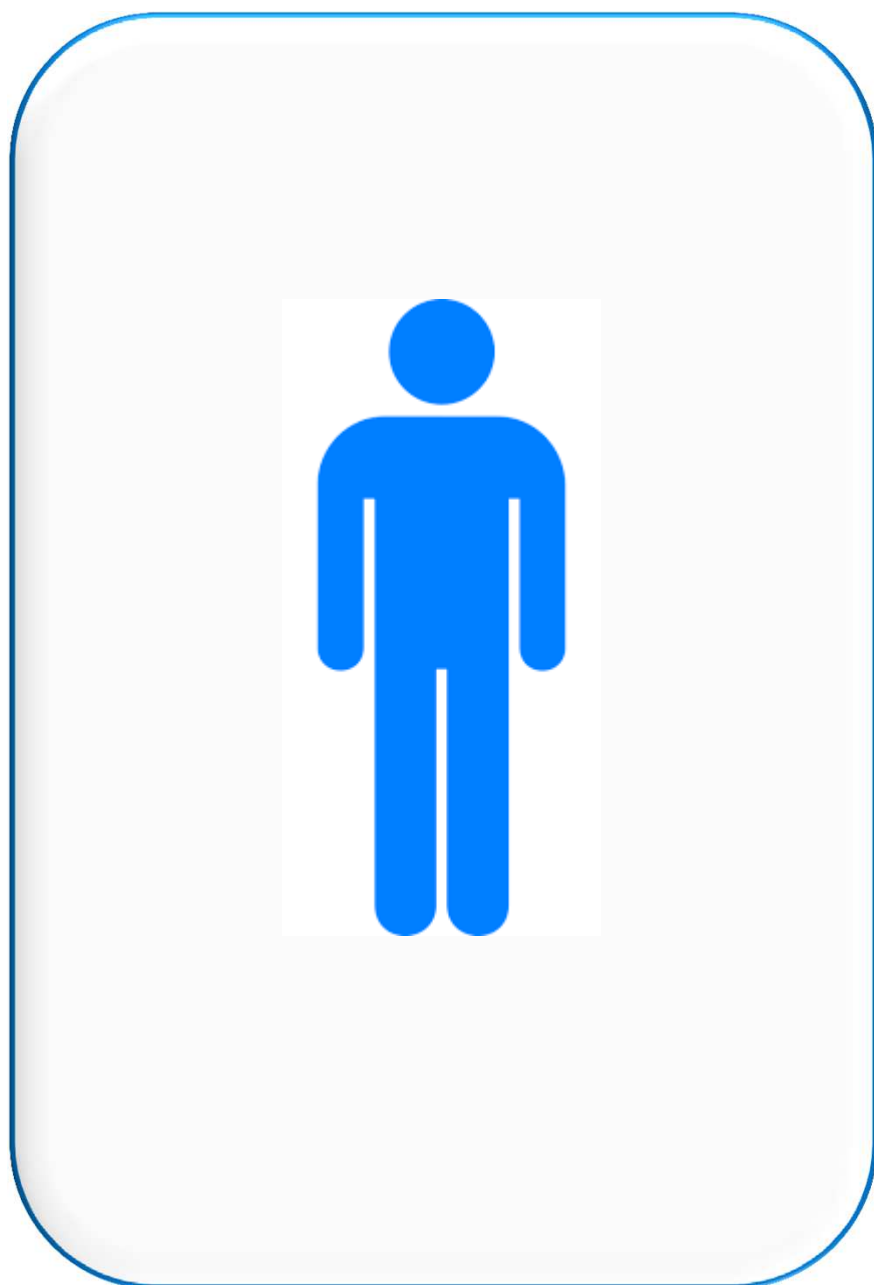
DOSE LIMIT

PHARMACODYNAMIC

-

**How the DRUG affects
the ORGANISM**







PHASE 1

**1st ADMINISTRATION
in man**

PHARMACOKINETICS

-

**How the ORGANISM
affects a DRUG**

**STUDIES
in
HEALTHY VOLUNTEERS**

UNIQUE DOSE
then possible
MULTIPLE ASCENDING
DOSE

1 PATIENT AT A TIME



SAFETY

A HUNDRED OF PATIENTS ENROLLED



EFFICACY

PHASE 2

MULTIPLE DOSE
over time

**Few Patients
Homogenous
population**

OPTIMUM DOSE

EFFICACY

TOLERANCE

**SEVERAL DOSES
TESTED**

-

**COMPARISON of
DOSES**

**LARGE NUMBER of
PATIENTS**

UNIQUE DOSE

PHARMACO- VIGILANCE

PHASE 3

PIVOTAL TRIAL

EFFICACY



**RANDOMIZED
CLINICAL TRIALS**

DOUBLE BLIND

VERSUS PLACEBO

REPRESENTATIVENESS

-

**POPULATION
TO TREAT**

EFFICACY

CLINICAL ENDPOINT

**THERAPEUTIC
INTEREST**

**BENEFIT/RISK
BALANCE**

**DOSSIER FOR
MARKETING
AUTHORIZATION**

**DEFINITION of the
CONDITION of the
MARKETING
AUTHORIZATION**

PHARMACO- VIGILANCE

PHARMACO- VIGILANCE

**SMALL NUMBER OF
PATIENTS**

TOXICITY STUDY

PHASE 4

POST-MA STUDIES

**RARE SIDE EFFECTS
DETECTION**

PHARMACO- VIGILANCE

COST/EFFICACY

**BENEFIT/RISK
BALANCE
IN
« REAL LIFE »**

**EVALUATION of the
USE of the DRUG :
MISUSE, GOOD USE**

REAL LIFE CONDITIONS

LARGE SCALE

**BETTER UNDERSTAND
THE THERAPEUTIC
EFFECT of the DRUG in
REAL LIFE CONDITION**

**OBJECTIVE :
OBTAIN THE MA**

EFFICACY

**SMALL NUMBER OF
PATIENTS**

PHARMACOKINETICS

-

**How the ORGANISM
affects a DRUG**