L’IMPLICATION DU MALADE EXPERT DANS TOUTES LES ETAPES DU DEVELOPPEMENT DES THERAPIES

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LE PATIENT PIONNIER

QUELQUES JALONS HISTORIQUES


- En prise sur de multiples sujets :
  - sensibilisation, formation, éducation, développement thérapeutique
  - concept du Patient Expert*

- Initiateurs d’un écosystème impressionnant : patients, familles, associations, scientifiques, cliniciens, universités, hôpitaux, instituts médico-éducatifs, entreprises de biotechnologie, laboratoires pharmaceutiques, agences réglementaires, investisseurs, organisations de prise en charge des frais de santé, instituts médico-éducatifs, éthiciens, acteurs de la santé digitale...

- Excellence et transversalité

* Loi Hôpital, Patient, Santé et Territoire (2009)
# Patients in Drug Development

## Basic Research
- Funding basic science
- Organizing patients
- Connecting to disease experts

## Discovery & Preclinical
- Bio-specimen, Natural History, Health Data collection
- Understanding and designing clinically meaningful endpoints

## Clinical Trials
- Educating disease community about the clinical trial process & participation
- Disseminating clinical trial news and information
- Supporting and developing the clinical network
- Understanding clinically meaningful endpoints
- Informing protocol feasibility – Clinical Trial Guidelines and impact on family

## Regulatory Processes
- Patient voice in the regulatory process

## Commercial (Drug on Market)
- Improving disease awareness and diagnosis
- Informing Clinicians
- Pharmacovigilance

*Source: Adapted from Global Genes*
ILLUSTRATION – ESSAIS CLINIQUES DE THERAPIE GENIQUE DANS UNE MSL*

- Soutien associatif aux premières heures du programme
- Histoire naturelle de la maladie :
  - Contexte règlementaire
    - Premier essai clinique international multicentrique de type NHS pour cette maladie
    - Comparateur pour un essai clinique de phase III aux US et EU
  - Contexte individuel
    - Enfants vulnérables et difficiles à gérer / Familles fragilisées ➔ Partenariat avec une association de patients pour la logistique
    - Tests cognitifs en endpoint primaire ➔ Programme spécifique re l’administration de tests neurocognitifs
  - Participation du patient / Génération de données pertinentes
    - Une donnée prédictive d’un bénéfice attendu par le patient
    - Endpoint primaire
    - Endpoints secondaires, y compris innovants (HTA)
- Création d’un Patient Advisory Board international
- Essai de phase III multicentrique

* MSL : Maladie de Surcharge Lysosomale
MERCI 😊