

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

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Karen Aiach

Karen Aiach is the founder, CEO and shareholder of LYSOGENE (LYS – FR0013233475). She has a child affected with a rare lysosomal storage disease.

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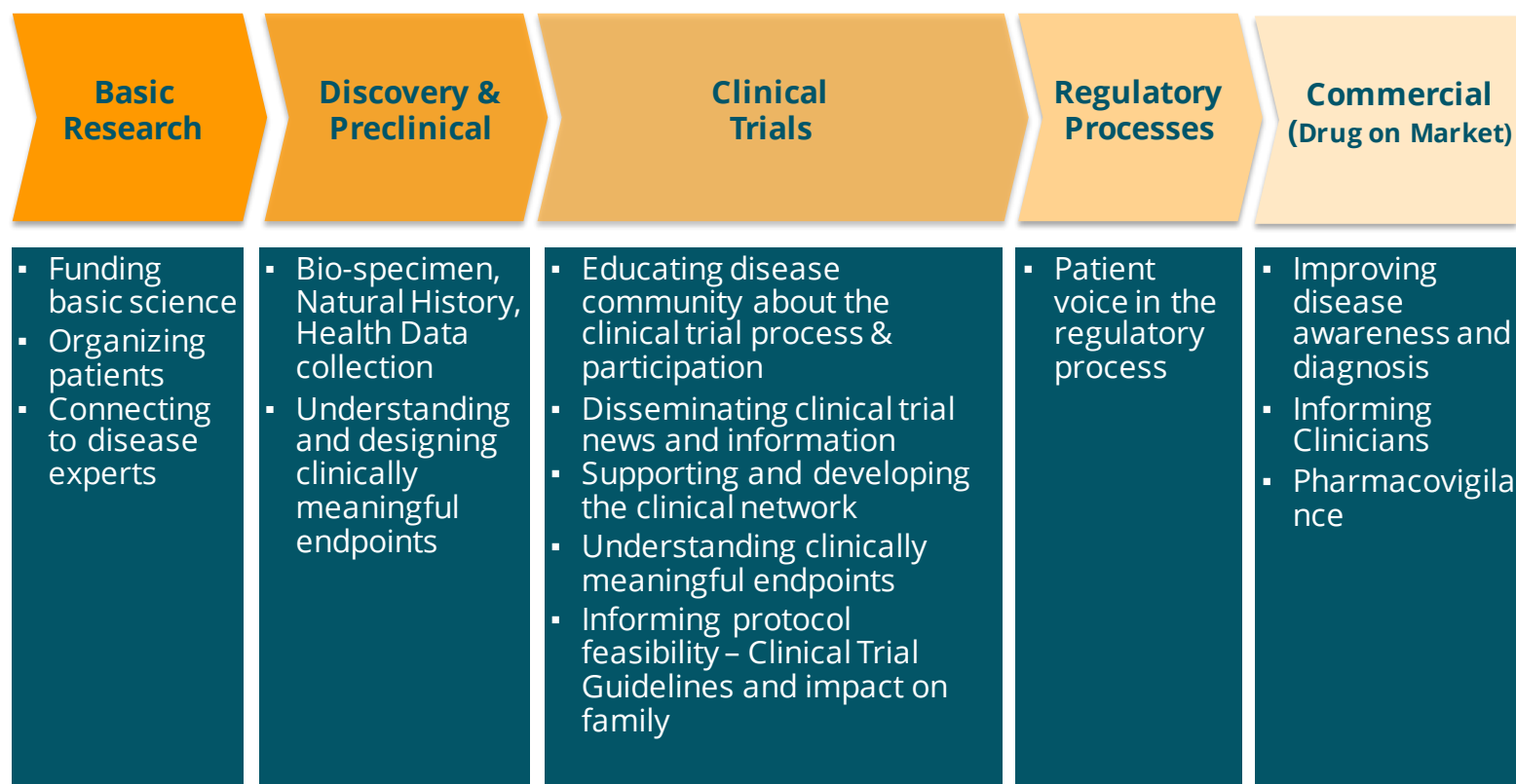
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## LE PATIENT PIONNIER

### QUELQUES JALONS HISTORIQUES

- Règlementations orphelines : USA (1983), Japon (1993), Australie (1997), Union Européenne (2000)...
- 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

## Patients in Drug Development



## 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 😊