Knowledge repository to enable patient-focus medicine development

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Need for public-private collaboration

- Funding
- Collaboration
- Standardization
- Trust
Objectives of the full project

- **Opportunity:**
  - There is currently no uniform process or understanding of capabilities of stakeholders to enable industry to engage patients in the drug discovery & development process

- **Objective:**
  - Develop and deliver a Patient Inspired Knowledge Hub (PIKH) that enables sharing of information and capabilities with and by users from patient groups, regulators, health authorities, academic medical centers, scientific organizations and industry.

- **Overall Goal:**
  - Make medicines development relevant to the real world patient and more efficient through systematic patient involvement.
  - Be the search tool for current global capabilities of stakeholders based on a defined patient engagement need which is accepted and continually refined by all stakeholders.
Today

Pharma 1 → Patients
Pharma 2 → Advocacy Groups
Pharma 3 → Regulators
Future

Pharma → Knowledge Repository → Pharma → Patients
Pharma → Knowledge Repository → Pharma → Advocacy Groups
Pharma → Knowledge Repository → Pharma → Regulators
Pre-competitive nature

- Best possible dialogue with patients is a key ingredient of developing products that will win in a competitive market place.

- It’s the right thing to do for the patients
Expected impact on the R&D process

1. **Holistic understanding of the patient life context** - includes co-morbidities, reaction to pricing/co-pays, Health Care Systems in which they participate, barriers to adherence, patient trade offs and decision making

2. **Health Technology Assessments** for payer acceptability

3. **Patient’s own Benefit-Risk assessment** of treatment outcomes versus their disease/situation - may result in potential prospective subgroup identification for regulatory approval

4. **Packaging design/market image** - cultural and regional acceptability is key

5. **Endpoint selection** - what endpoints and messages about products resonate with patients and meet their expectations?

6. **Clinical trial design/ protocol** - feasibility assessment; how many visits, tests, specialist visits

7. **Clinical trial recruitment optimization** — (plus diversity focus) ; input on standards, communications before, during and after trials; within trial patient communities

8. **Health Literate Patient labeling** -

9. **Patient education materials and health care apps** - throughout life cycle
Suggested architecture of the project

- Specification & Planning
- Patient Advocacy Engagement
- Regulatory Engagement
- Academic Engagement
- Technology Platform & Tools
- Pilots
- Sustainability and Business Model
- Dissemination
- Management
Expected contributions of the applicants

- **Domain Expertise**
  - Clinical Knowledge
  - Regulatory Affairs
  - Patient Advocacy
  - Many more

- **Functional Expertise**
  - Patient Focus Groups
  - Running pilots in settings of care
  - User Centred Design
  - Business Process/Workflow Modelling

- **Service Provider/Execution**
  - Product Management
  - Project Management
  - Technology Development
  - Protocol Development
  - Integrator
  - Commercialization and Business Development
  - Many more
Expected (in kind) contributions of EFPIA members

- Domain Expertise
  - Drug Discovery all the way to Commercial
  - Support services (scientific modelling, sales, supply chain, etc)
  - CIO & CMO organizations
- Network Access
  - Industry
  - Academia
- Execution (FTE)
  - Product development
  - Project management
  - Domain expertise (Clinical, regulatory, commercial, etc)
- Existing Assets
  - Technology and Platform
  - Knowledge, Protocols, Tools, etc
What’s in it for you?

- Advance the state of Science directly and indirectly
- Translate research and academic findings into practice
- Developed bio-informatics platforms and tools
- Create a better dialogue between patients and industry
- Provide transparency into menu of best practices
- Leverage this project to integrate other Patient centred initiatives
- A place for stakeholders to gather and have an exchange
- Practical experience with working multiple pharma companies under one roof
- Funds to finance SME growth
- Build infrastructure that will lead to commercialization opportunities
Key deliverables of the full project

- Identifying the appropriate points in time to interact with patients for development of medicine, including: risks and benefits of interactions, required capabilities, anticipated enabling changes in regulatory affairs and more.

- Standardizing a framework to be used for patient engagement in medicine development.

- Providing the ecosystem and mechanisms for stakeholders, for example pharmaceutical companies and patient advocacy groups to discuss and share frameworks, methods and knowledge.