An Advocacy Perspective

- Why develop drugs for Rare Disease?
- What is the thought process of advocacy around access?
- What is the public missing beyond the cost and headlines?
- Why should we think about this differently?
- Possible solutions? Next Steps?
Why Develop Drugs for Rare Diseases?

About 7,000 Distinct Rare Diseases Recognized Today

350 Million People Globally Are Fighting Rare Diseases
- 1 In 10 Americans Suffer From A Rare Disease, That’s ~30M People

Most Are Chronic And Many Are Progressive
- Approximately 50% Affect Children & 30% Die Before The Age Of 5

Only ~ 350 Have Identified Treatments
What is the thought process of advocacy around access?
First…

Advocacy Groups Are Not Created Equally

Each Rare Disease & Each Rare Disease Patient Community is Different

Therefore, each group must take into consideration
- Severity of Disease?
- Current treatment landscape and what treatments are available?
- What level of risk will they accept?

All of this drives the push for access
Advocacy’s Role Is to Facilitate Access From Research to Commercialization

Support
- Family Support Workshops
- Family Networking

Research
- Scientific Updates

Care
- Professional Medical Provider Education

Research & Clinical Trial Updates

Patient Care Workshops

Cross Discipline Networking
Facilitating Clinical Trials and Drug Development

- Reduce Risks for Investment
- Support with Recruitment and Design
- New Trial Outcomes and Meaningful Endpoints
- Providing Patient Perspective to FDA
  - “Voices of the Patient Report”
  - Patient meetings
  - PFDD meeting
- Patient focus groups and surveys
  - Clinical trial participation/expectations/barriers
  - Meaningful benefit and Benefit/Risk
  - Impact on daily living
What is the public missing?
Perfect Storm

- **Benefit – risk assessments**
- Players move in isolation – multi stakeholder alignment?!
- **Current model for healthcare and medicines development not sustainable**
- **Tissue banking**
- **Big Data**
- **Value demonstration**
- **The regulator no longer final hurdle**
- **Trust & transparency**
- **Patient centric models as an answer?**
- **More networked and educated patients**
- **New societal contract required?**
- **High cost pressures on health care systems**

*amyloidosis research consortium*
The economic problem is to match limited resources to unlimited wants and needs.
Access Is Not Price

- Access is the most important factor to rare disease patients.
- Most patients only get access to a drug once it has been FDA approved.
- FDA is starting use regulatory flexibility to approve therapies quickly, because patients are waiting for access.
- Approval doesn’t always lead to access.
  - Patients face challenging reimbursement hurdles to gain access to approved therapies.
Navigating Reimbursement

- Price is still important to patients and patient advocacy groups, but price transparency and a clear understanding on how patients will get access is critical.
- Most often it is the patient, family, and clinician that will take on the burden of reimbursement issues and appeals processes.
- Example: Payer restrictions versus drug label restrictions
  - SPINRAZA was approved for the treatment of SMA, no type restrictions, yet payors imposed their own type restrictions and benefit timelines
    - Payers have limited disease understanding
What should we think about differently?
Role of Patient Led Foundations Across Drug Development

Valuable role in developing tools and support to accelerate drug development

Reduce uncertainty over the benefit-risk-value
Patients are experts in their disease, and properly engaged can play a vital role in all stages of drug development.

Embedding patient preference data at every stage:

- Study design population, endpoints, B/R acceptability
- Invention + Prototyping
- Pre-Clinical
- Clinical
- Regulatory Decision
- Product Launch
- Post-Market Monitoring

Unmet need, prioritization & Foundation support:

- Discovery + Location
- Regulatory evaluation
- Real-World data collection e.g. registry
Evidence Generation to Support Market Access

De-Risking & Improving Value

Better Research for Better Outcomes
Building Programs to Support Drug Development

Taking Advantage of Existing Opportunities With FDA

- Drug Development Round Table
- Guidance for Industry on Drug Development
- Biomarker Development
- Patient Focused Drug Development Meeting
- Patient Voice Publication
Creating A Value Proposition

- Disease impact and clinical benefit to patients needs to be strongly linked
  - Justify rationale for selected clinical endpoints and their relevance to patients
  - Long-term benefits clear

- Demonstrate that the price is reasonable given:
  - Small patient population
  - Severity of disease
  - Level of unmet need
  - Development costs

- Potential decrease in other costs due to better health and functioning
  - Less need for caregivers
  - Decrease in other medications
  - Ability to work
Possible Solutions?

Change the Approach
Incorporate the Patient Perspective from the Beginning

Start and End with the Patient in Mind
Strengthen Advocacy with Industry Expertise:
Increase Industry Participation in Research Consortiums
Incorporating patient perspective in funding decisions for rare disease treatments: a review of international payer systems

C Falace, R Sur, J Ballyzani, A Hutchinson
Dolun Ltd, London, UK

Background and Objectives
- Healthcare payers are increasingly aware of the need to engage patients at the earliest stages in the development process of new disease treatments.
- This article reviews the benefits of incorporating patient perspectives in the decision-making process of new disease treatments.

Methods
- A qualitative study was conducted to understand the perspectives of patients and caregivers on rare disease treatments.
- The study included interviews with patients and caregivers to gather insights on their experiences and needs.

Results
- Patients and caregivers emphasized the importance of patient-centric approaches in the development of new disease treatments.
- They highlighted the need for early involvement of patients in the decision-making process.
- The study also revealed the importance of education and awareness programs to inform patients and caregivers about the disease and available treatments.

Implications for Future Research
- Further research is needed to understand the role of patient perspectives in the decision-making process of new disease treatments.
- The integration of patient perspectives in the development of disease treatments can help improve patient outcomes and satisfaction.

Educate payers long before drug approval
- Burden of disease on patient and caregiver
- What do patients hope to see in a treatment
- Education on patient perspective needs to be done across all stakeholders – Pharma, Regulators and Payers
How do we Synchronize Expectations Across FDA, Payers and Industry

FDA: Payer Communication Task Force
A step in the right direction?

About FDA

Payer Communication Task Force

Background

CDRH established the Payer Communication Task Force to facilitate communication between device manufacturers and payers to potentially shorten the time between FDA approval or clearance and coverage decisions. By communicating earlier, manufacturers may design their clinical trials to produce the data required for regulatory approval or clearance and positive coverage determinations, which may expedite patient access.

Payers include public payers such as Centers for Medicare & Medicaid Services (CMS), private health plans, health technology assessment groups, and others who provide input into coverage and reimbursement decisions.

CDRH’s Center for Devices & Radiological Health (CDRH) evaluates the safety and effectiveness of medical devices for use in the U.S. Usually after FDA approval or clearance, other organizations—for example, public and private organizations that pay for health care (payers) and the professionals who provide health care (providers)—decide whether to cover, pay for, or use a device. Often, the data submitted by medical device manufacturers to demonstrate safety and effectiveness to the FDA may not include data needed by payers to make coverage determinations. As a result, after FDA approval or clearance, there may be a delay in coverage, payment and use decisions that may ultimately delay patient access to medical devices.

Opportunities to Obtain Public Payer Input

The opportunities for collaboration with a public payer, CMS, are outlined below. CDRH hopes to further expand the opportunities to include collaborations with other public payers in the future.

Parallel Review

IONIS PHARMACEUTICALS

[Image of children in a park, each engaged in different activities.]

[Image of a woman and a child walking on the beach.]