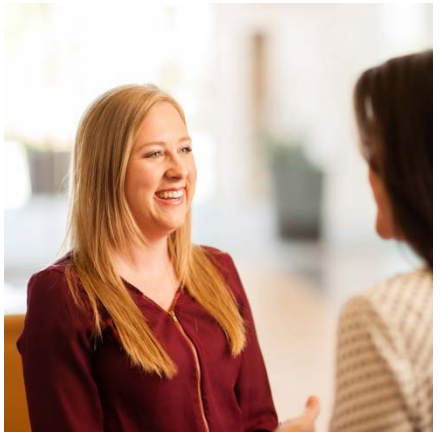

Developing Drugs for Rare Diseases: Patient Advocacy's Perspective

Kristina Bowyer

Executive Director, Patient Advocacy

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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

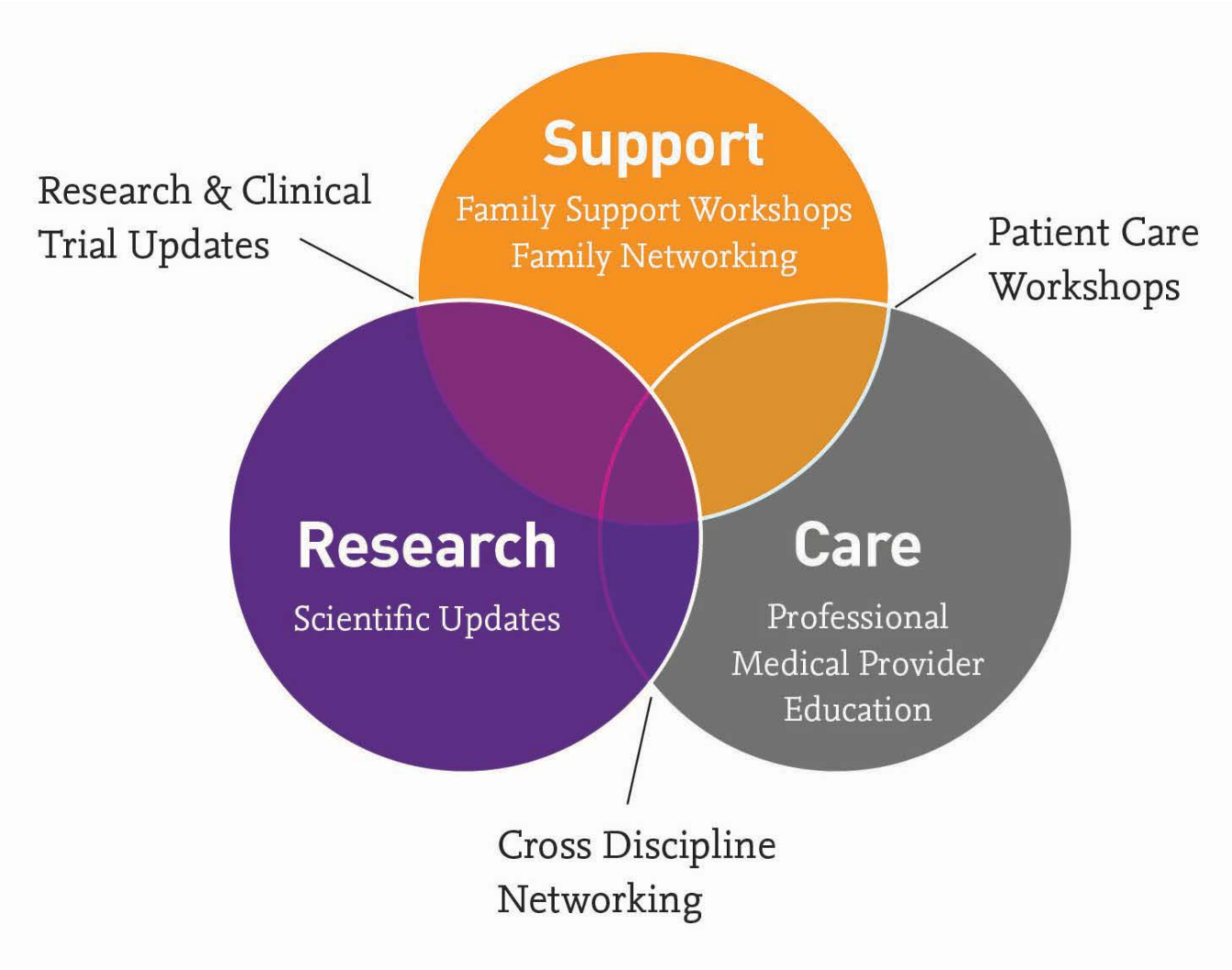
Therefore, each group must take into consideration

- 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

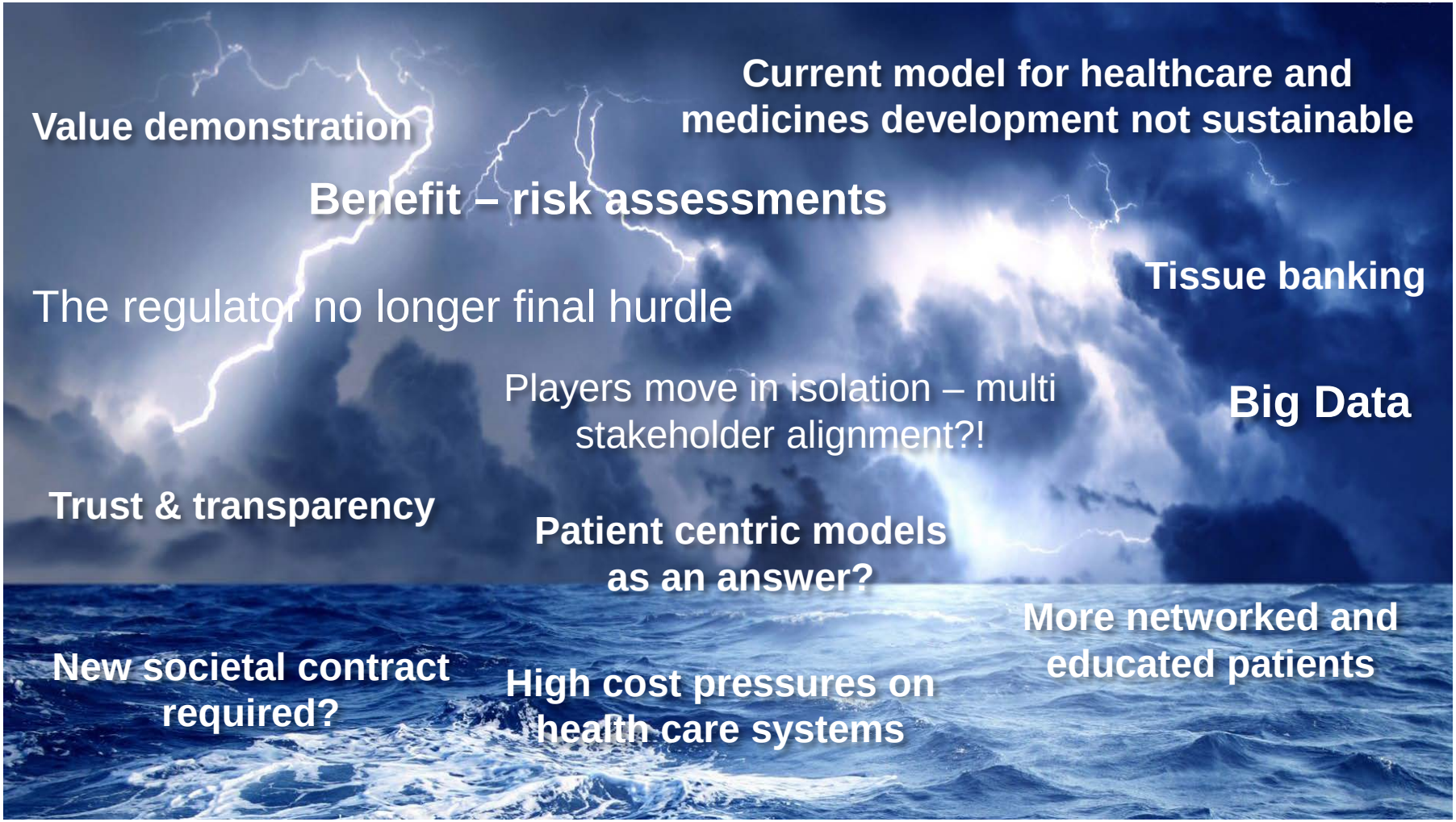


Facilitating Clinical Trials and Drug Development

- 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



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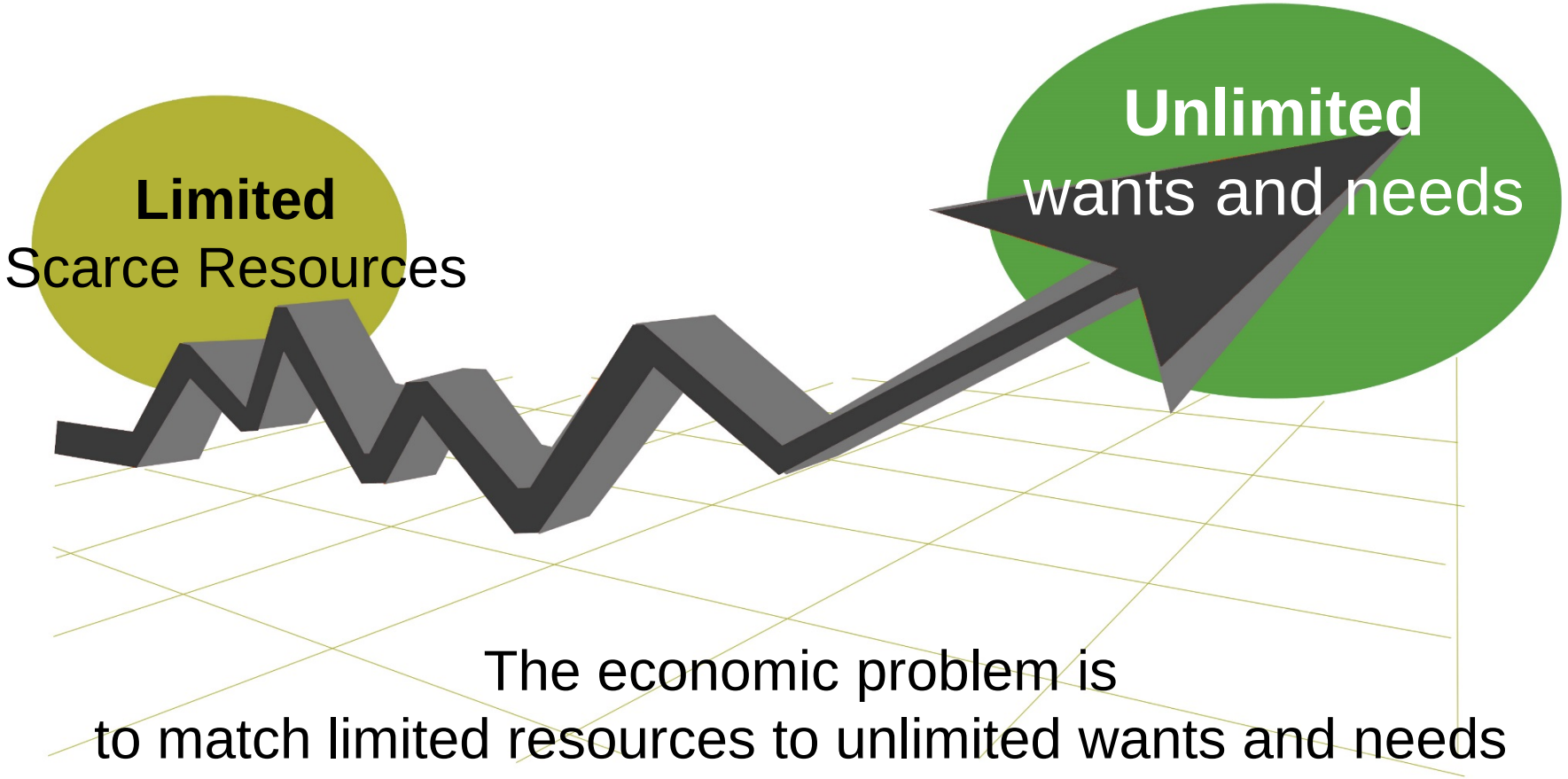
Resources



The economic problem is
to match limited resources to unlimited wants and needs

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Access Is Not Price

- Access is the most important factor to a rare disease patients
- Most patients only get access to a drug once it has been FDA approved
- FDA starting use the 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

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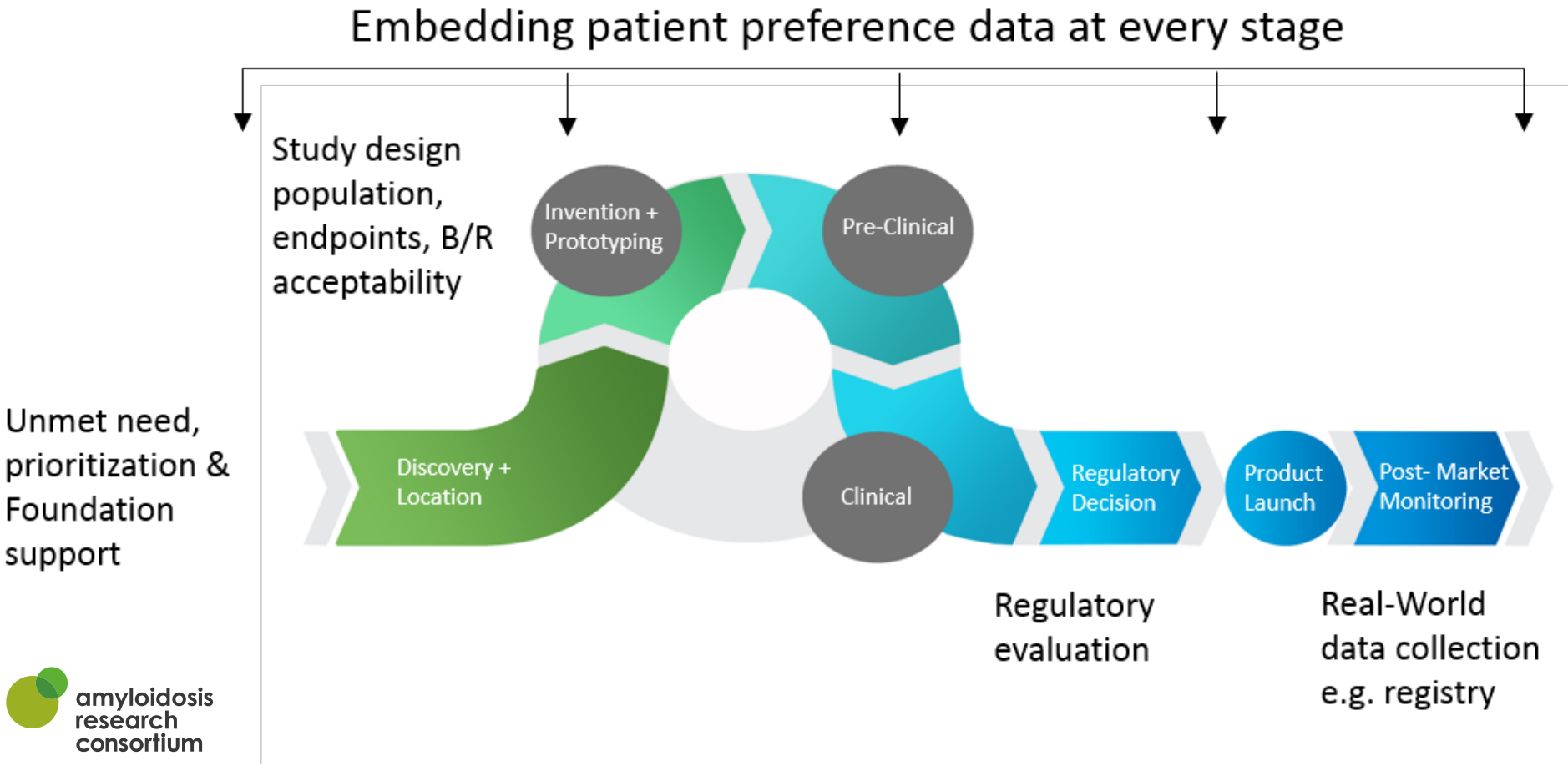
Role of Patient Led Foundations Across Drug Development

Patients are experts in their disease, and properly engaged can play a vital role in all stages of drug development



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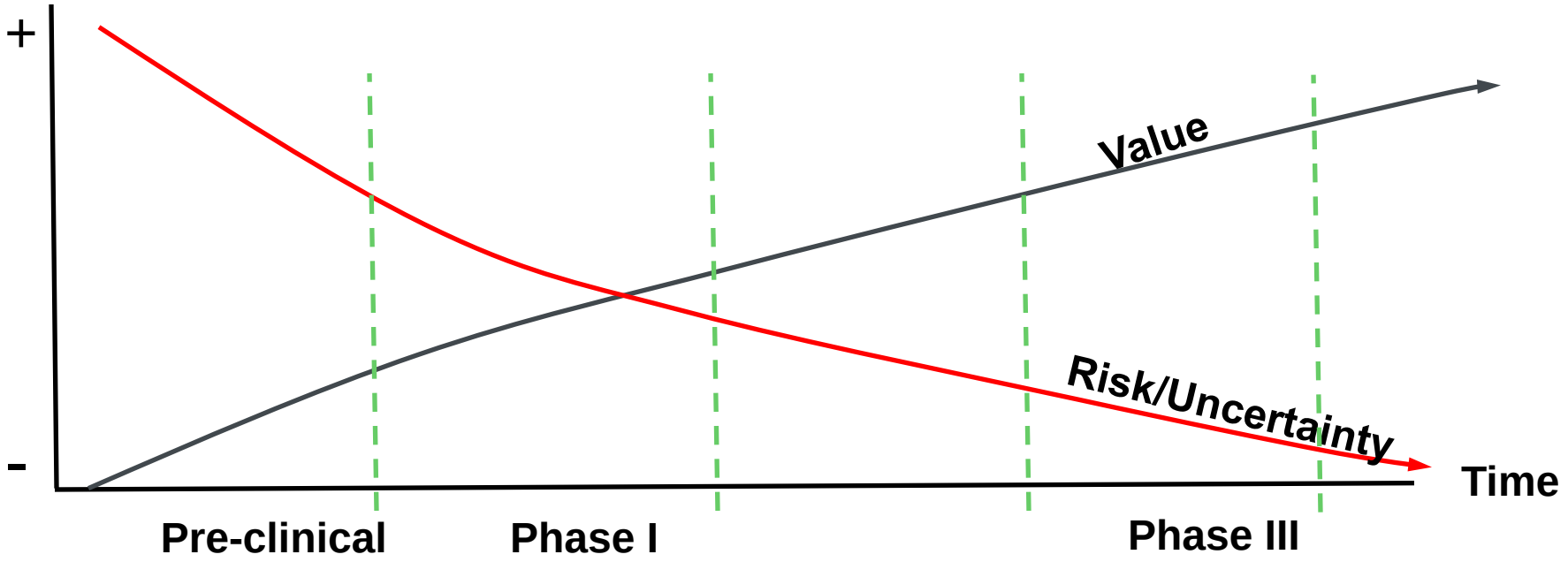
Evidence Generation to Support Market Access

De-Risking & Improving Value



Better Research for Better Outcomes

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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

Supporting Payer Needs to Make Better Decisions About Coverage of Rare Disease Treatments

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

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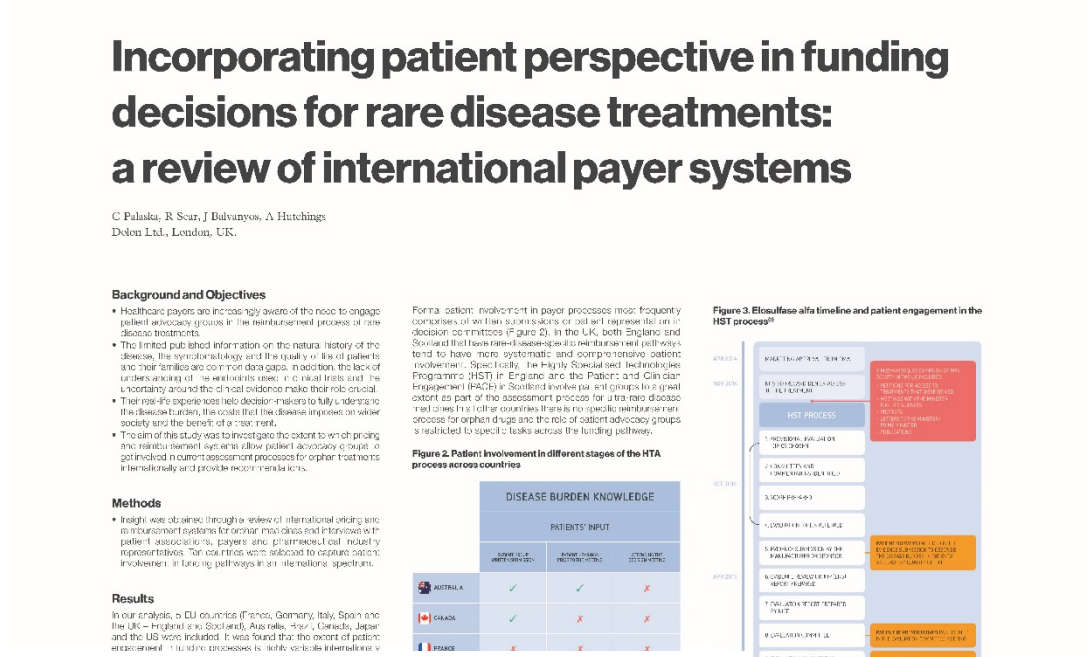
Incorporating Patient Perspective in Funding Decisions for Rare Disease Treatments



Incorporating patient perspective in funding decisions for rare disease treatments: a review of international payer systems

C Palaska, R Sear, J Balvanyos, A Hutchings
Dolon Ltd., London, UK.

Background and Objectives

- Healthcare payors are increasingly aware of the need to engage patient advocacy groups in the reimbursement process of rare disease treatments.
- The limited published information on the natural history of the disease, the symptomatology and the quality of life of patients and their families are common data gaps. In addition, the lack of understanding of the endpoints used in clinical trials and the uncertainty around the clinical evidence make their role crucial.
- Their real-life experience can help decision-makers to fully understand the disease burden, the costs that the disease imposes on wider society and the benefit of a treatment.
- The aim of this study was to investigate the extent to which pricing and reimbursement systems allow patient advocacy groups to get involved in current assessment processes for orphan treatments internationally and provide recommendations.

Methods

- Insight was obtained through a review of international pricing and reimbursement systems for orphan medicines and interviews with patient associations, payers and pharmaceutical industry representatives. Ten countries were selected to capture patient involvement in funding pathways in an international spectrum.

Results

In our analysis, 5 EU countries (France, Germany, Italy, Spain and the UK – England and Scotland), Australia, Canada, Greece, Japan and the US were included. It was found that the extent of patient engagement in funding processes is highly variable internationally.

Formalisation: involvement in payer processes most frequently comprises of written submissions or patient representation in decision committees (Figure 2). In the UK, both England and Scotland that have rare disease-specific reimbursement pathways tend to have more systematic and comprehensive patient involvement. Specifically, the Highly Specialised Technologies Programme (HST) in England and the Patient and Clinician Engagement (PACE) in Scotland involve panel groups to a great extent as part of the assessment process for ultra-rare disease medicines. In all other countries there is no specific reimbursement process for orphan drugs and the role of patient advocacy groups is restricted to specific tasks across the funding pathway.

Figure 2. Patient involvement in different stages of the HTA process across countries

	DISEASE BURDEN KNOWLEDGE	PATIENTS' INPUT	
AUSTRALIA	✓	✓	✗
CANADA	✓	✗	✗
FRANCE	✗	✗	✗

Figure 3. Elosulfase alfa timeline and patient engagement in the HST process[6]

mendations

gagement has been seen to improve the quality of funding making, particularly in respect to improving prevalence y in enrolling, the relevance of clinical trial endpoints and ng the impact of the disease and treatment on the lives s and their families.

the effectiveness and the value of patient involvement proves further through:

on of patient advocacy groups on payer systems
rce-sharing with other patient associations
of patient surveys to provide insight into the disease impact sible representation in the HTA bodies that assess orphan of an overarching patient association

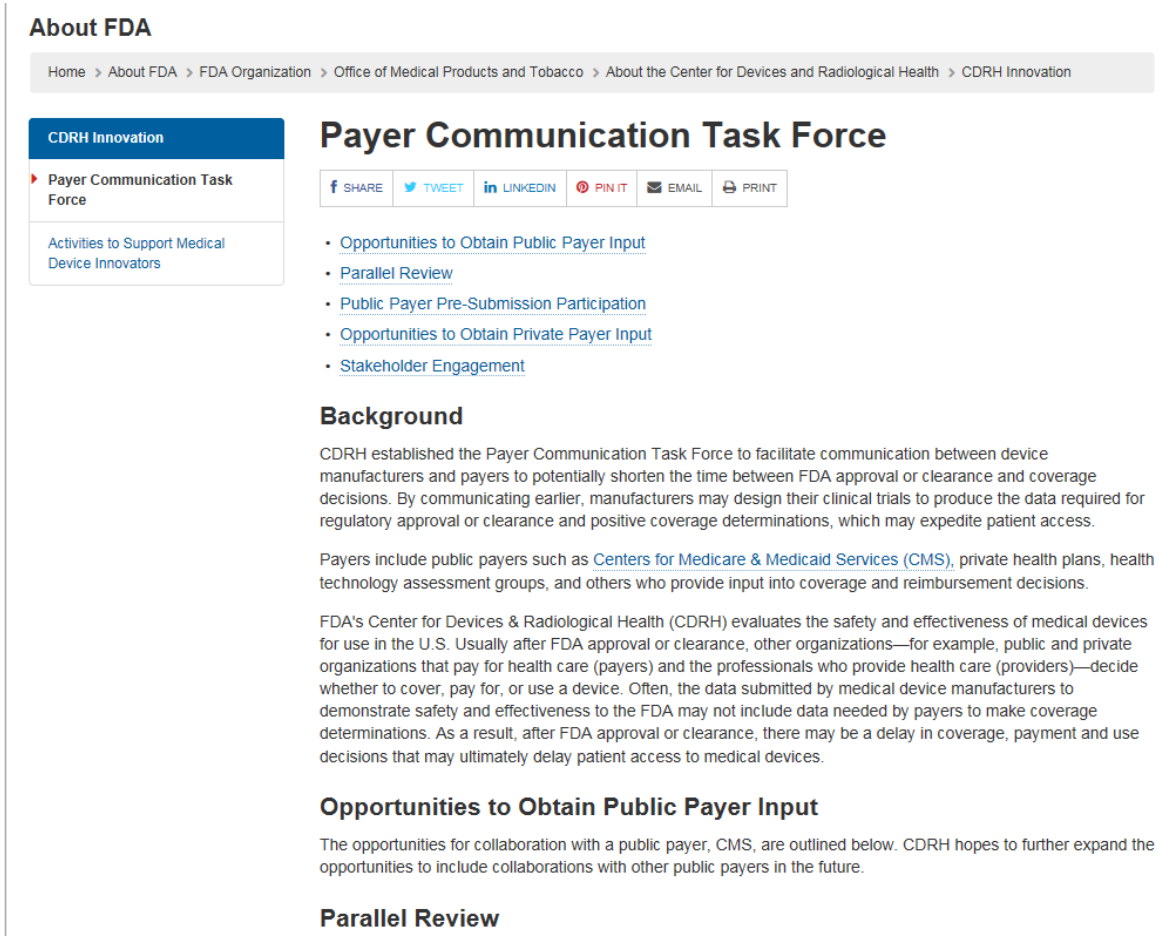
sions

clear need for more systematic patient involvement in decisions for rare disease treatments. Although the extent the patient input influences these decisions may vary countries, patient representatives (and individual patients) vital role to play in the understanding of their diseases terpretation of the benefit of new treatments. Payer decision hould incorporate both general rare disease patient ation and disease-specific patient input.

- 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

Home > About FDA > FDA Organization > Office of Medical Products and Tobacco > About the Center for Devices and Radiological Health > CDRH Innovation

CDRH Innovation

- Payer Communication Task Force
- Activities to Support Medical Device Innovators

Payer Communication Task Force

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- Opportunities to Obtain Public Payer Input
- Parallel Review
- Public Payer Pre-Submission Participation
- Opportunities to Obtain Private Payer Input
- Stakeholder Engagement

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.

FDA'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

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