amyloidosis research consortium

Drug Development in Rare Diseases (Amyloidosis): Consortium Perspective

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The issues

amyloidosis research consortium

Unmet need: Significant therapeutic unmet needs – urgent need for new treatments

The system: slow, bureaucratic

No clear strategy: no blueprint/strategy for clinical trials, prioritization and strategy

Lack of collaboration: stakeholders were working and thinking isolation

Uncertainty: Data uncertainties and weak value proposition for decision-makers beyond the regulator

Resources: limited resources not being maximised

Not patient-focused in any way. Patients are not getting the treatments they need.

Case study

- First novel agent in myeloma
- Received its European licence in 2004 as a **monotherapy** in relapsed myeloma patients
- Myeloma patients in England and Wales did not get access to Velcade until 2007
- Major problem with the trial design and high price, so NICE issued negative guidance

Consortia Model

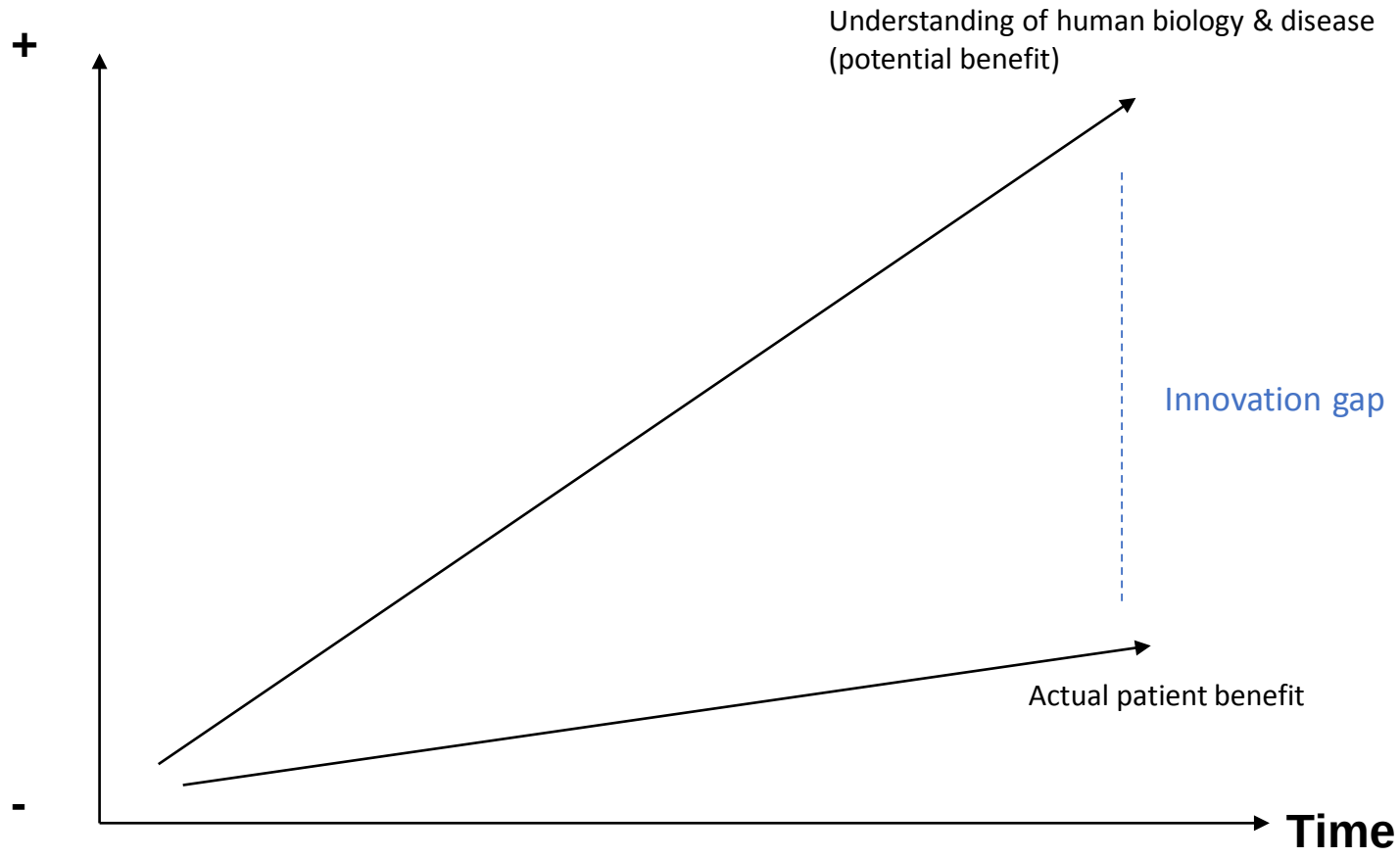
- "The role of research charities and patient organisations has evolved from a primary emphasis on grant funding to a driving force that is advancing scientific development and leading cutting edge patient-centred research."

WHEN IT COMES TO THE SEARCH FOR CURES, NO ONE GOES IT ALONE. Getting new medical products from discovery to patients requires all sectors—academia, industry, government, clinical care, non-profits, and philanthropy— to work together throughout the research and development process. But collaboration is a complex endeavour, and integrating the right partners is far from easy.

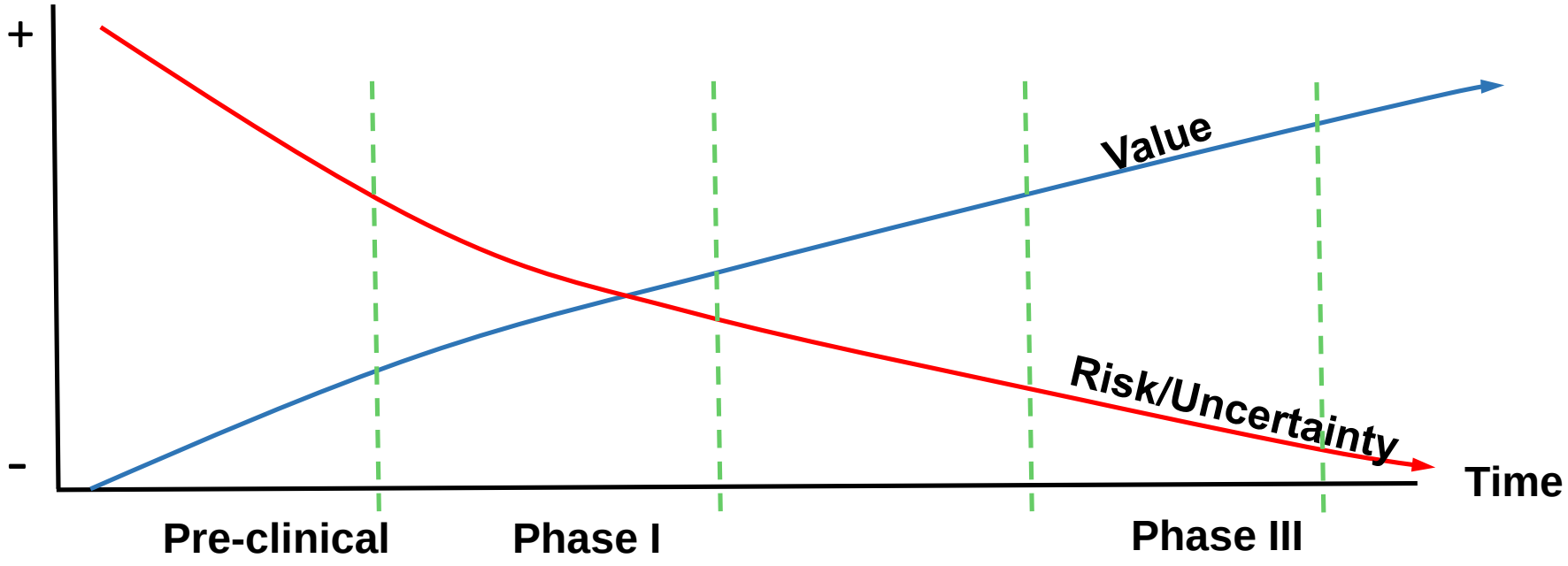
Trial Design

You can‘t fix mistakes in trial design leading to bad data, if you address them when reimbursement decisions are being made

Innovation Gap



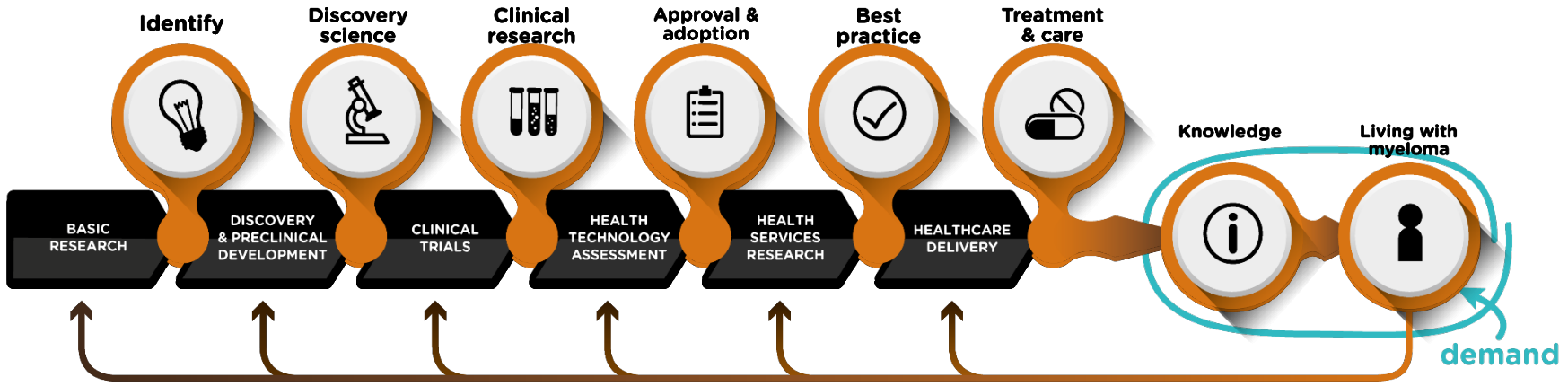
Derisking, improving value proposition



Better Research for Better Outcomes

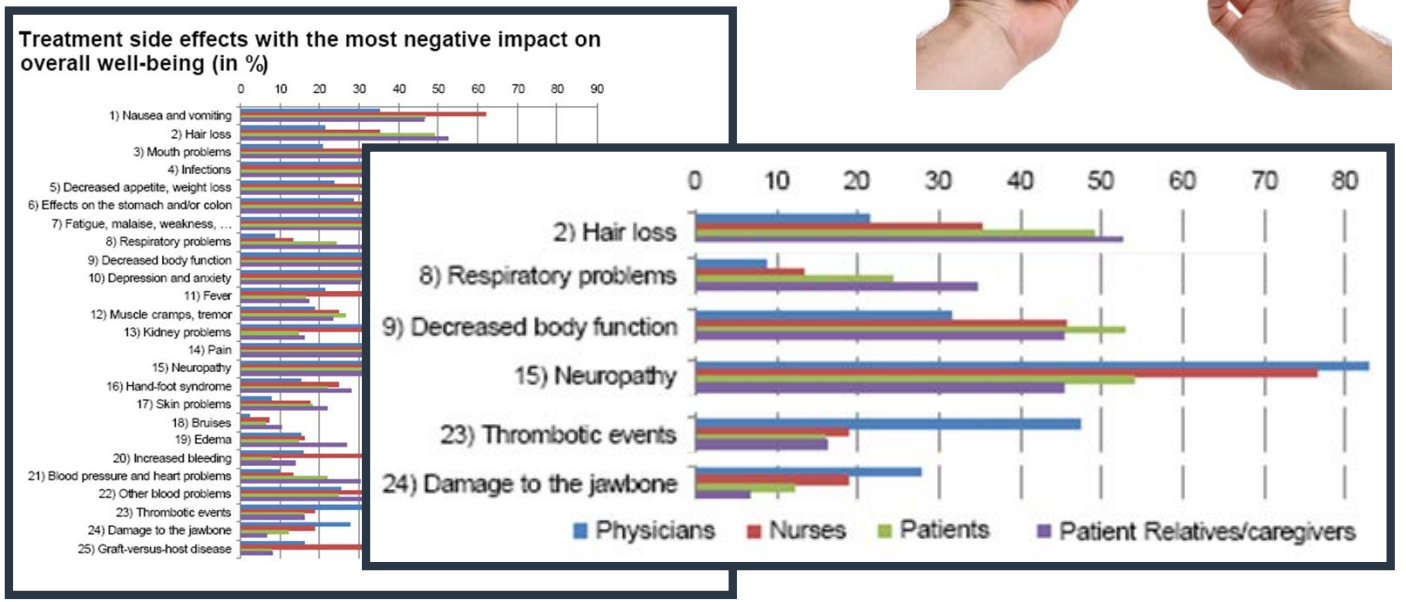
The model

- Demand-driven reverse translational model – from patients to discovery and back again
- Aligning agendas, rewards and incentives around a common goal – patients
- Accelerating the discovery, development of and access to the treatments and care patients need in a collaborative, integrated model



Outcomes that matter to patients

- Patients are different and the outcomes that matter to them and the criteria that they apply in regard to the potential benefits and risks of treatment are very different
- What is more, they are likely to change over the course of time in response to a range of things i.e. disease-related but also personal factors
- Holistic needs assessments and validated benefit/risk, QofL and PRO tools are needed through the course of any disease
- The outcomes that are important to patients may not be the same outcomes that are important to doctors or carers
- A strong therapeutic alliance is key



Capturing patient-centered outcomes

- Quality of life tools
- Patient reported outcome measures
- Patient preferences for treatment in the context of potential benefits and risks

Becoming increasingly important to articulate value proposition and to differentiate from other treatments especially where the clinical benefit is marginal.

The clinical trials system is "broken" and there needs to be new ways to collect and utilize patient data, Janet Woodcock, director of FDA's Center for Drug Evaluation and Research, told a workshop at the National Academies of Sciences, Engineering, and Medicine.
(September 20, 2017)

https://endpts.com/fdas-janet-woodcock-the-clinical-trials-system-is-broken/

Building Programs to Support Drug Development

- Research Strategy Blueprint
- Patient Focused Drug Development Meeting
- Patient Voice Publication
- Guidance for Industry on Drug Development

- Expert Advisory Committees
- Patient Review Panels
- Natural History Study
- Evidence generating trials

Case Study

Myeloma UK Clinical Trial Network (CTN) MUK *eight* phase IIb clinical trial to reduce uncertainty:

Collaboration with Takeda (Millennium) Pharmaceuticals

Critique of regulatory clinical development plan identified risks that would likely lead to uncertainty

Designed a phase IIb Myeloma UK CTN study to generate evidence to mitigate risks and improve value proposition submitted jointly to NICE Scientific Advice as part of pilot

Concept of the hybrid study was born

Summary

- It should start and end with the patient
- We need empirical data on what matters to patients
- Patient centred outcome methodology and tools need to be imbedded across the whole drug development, drug approval and drug reimbursement continuum and indeed in the clinic
- We need to improve clinical research and establish better alignment between the data needs of regulators and those of HTA bodies, payers and patients
- Better data is key to improving access
- Evolving role of patient/research organisations is changing the business
- Collaborative models, syndicated drug development and taking a prioritised and strategic approach will be a key to success
- Role of advocacy to hold the community to account