

The politics of rare diseases and orphan drugs

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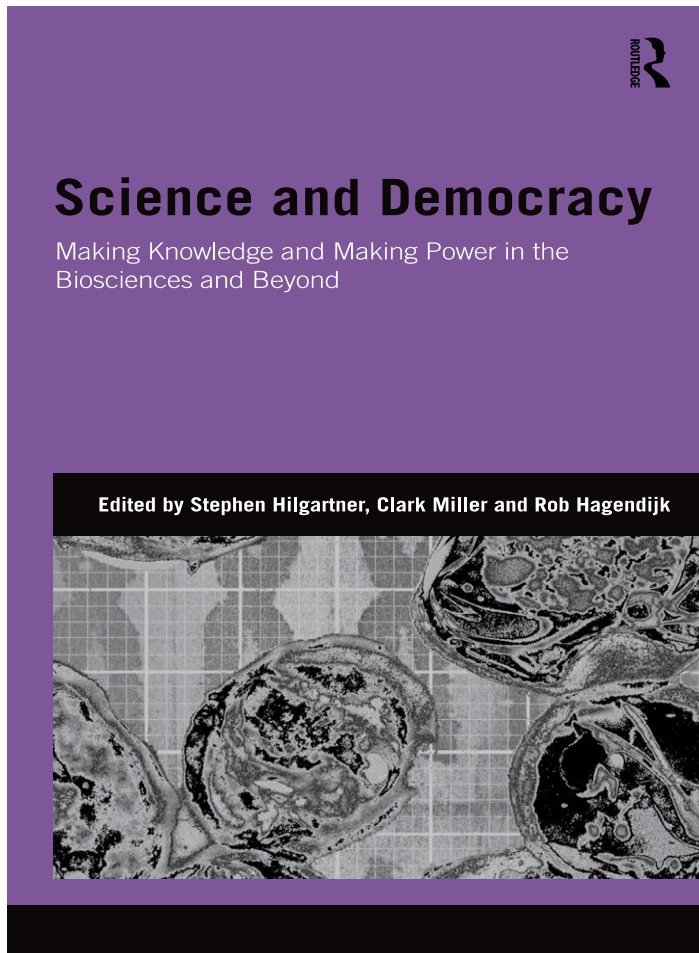
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Science and Technology Studies



- Key assumption: Science and society are made together
- Co-production analysis
- Micro/macro-perspectives should be combined
- Discourse coalitions and sociotechnical imaginaries drive change processes
- Life sciences: a key domain

The Dutch Pompe – Fabry Controversy

- August 2012 – 2013
- Leaked draft advice CVZ (Health Care Insurance Board → government): Stop reimbursement for the recently admitted new drugs for the two diseases mentioned
- The argument: Low cost-efficiency/ low efficacy
- Wider context: Fear for unsustainability of public health care system due to rising costs → tough and difficult decisions are required

Reframing the issues

First:

- Hype, political turmoil and stalemate

Initial response Ministry:

- Stick to procedure for advice and consultation, i.e. buy time
- Start discussions with industry about price cuts

Reframing the problem:

- Support European collaboration at the level of the diseases concerned
- Endorse EU policy making
- Renew health insurance arrangements for a limited time

Shifting configurations, conflict and governance

- The Pompe - Fabry Controversy as a case study
- Broader: European and international developments in biosciences and health care

To analyse:

- Changing problem definitions and perceptions
- Shifting power configurations and networks
- Actor-networks: Scientists, doctors, patients, industry, health insurance, politicians, administrators, non-human actors
- Redefining interests and solidarities
- Complex dynamics of change

EUROPE



Project Europe

- From common market to EU
- Developing a single market for pharmaceuticals...
- Beyond the common market
- Optimism: The Lisbon Agenda (2000- 2010). Knowledge economy and ICT
- Social Europe: (in)equality and citizen's rights
- Advances in the biosciences and medicine, changing work patterns in research

- Pessimism: The financial crisis, economic depression crisis and the rising multipolar world
- Aging Europe, demography
- EU expansion and fragmentation
- Anti- European uproar: A crisis of legitimacy

- *And yet: High legitimacy for rare diseases as an EU policy domain (added value)*

Health: A rising EU policy frontier?

- Path dependency: From single market to advanced European integration and citizens rights, social Europe
- Versus national sovereignty
- Experimental policymaking: The open method of coordination (OMC)
- The usual structural dilemma's and constraints in EU policy making: delays and advances
- Example: Directive patients' rights and cross-border healthcare

- Rare diseases form an experimental and strategic policy space (compare environmental policy, 1970s)
- The importance to form a strategic agenda and consensus (against adhocism and particularism)
- Building/strengthening a discourse coalition and joint imaginary

A strategic discourse coalition on rare diseases?

- The future of (EU) public health depends on a rethinking and renegotiation of the boundaries between state, market and civil society
- Orphan drugs and rare diseases together potentially form a key learning space for each dimension. Modest size of domain, modest risks, much opportunities to learn and test policy arrangements
- Criss-crossing political, economic, legal and scientific/medical challenges and shifting stakes and interests
- Work in this learning space might lead to a strong discourse coalition and corresponding sociotechnical imaginaries in favour of change
 - Develop agenda
 - Collaborate
 - Try ideas
 - Mobilize patients, professionals
 - Public opinion
- But Also to risks, conflict, policy fragmentation, techno-bureaucracy, short-termism → decline in legitimacy

So....?

Thank you!