Cooperation in the Field of Rare Diseases
A Social Science Perspective

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Introduction

- As unanimously claimed within the rare disease community, **cooperation between all stakeholders** is a necessity in order to tackle these diseases and improve the difficult situations and experiences faced by patients and their relatives. Health professionals, public health authorities, scientists, pharmaceutical firms, or obviously patients and relatives' associations, have an active part to play in the collective projects that are currently being developed around Europe. But **putting cooperation into practice does not always go unhindered**. Drawing on a research in social & political sciences, this poster analyses the drivers and effects of cooperation in the field of rare diseases. After the presentation of the research background and methodology, the poster highlights some significant findings and contributions of a social science perspective for the rare disease community.

Research & Methodology

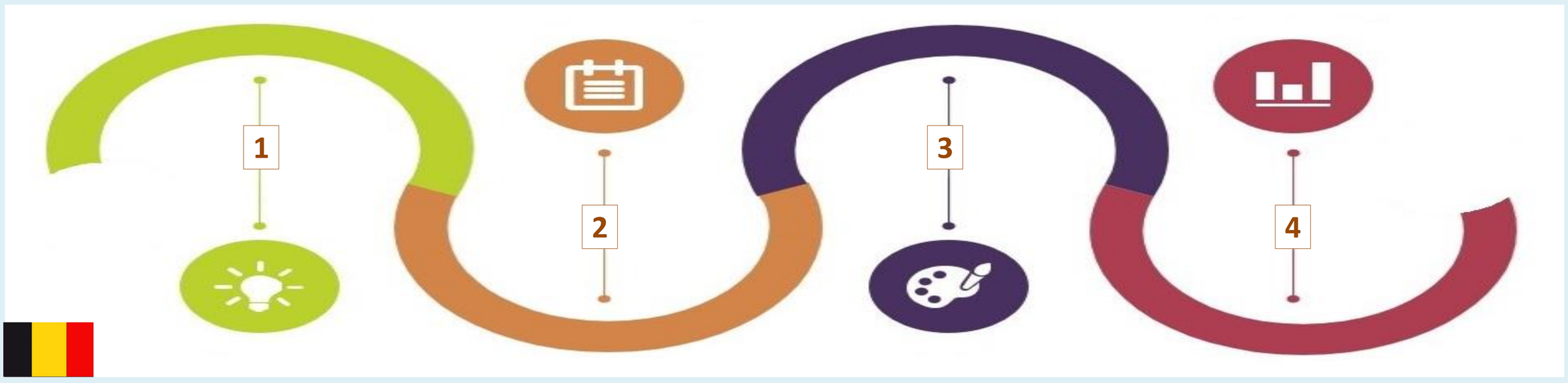
- The research (2015-2018) studies **the social and political dynamics which drive the contemporary field of rare diseases** in Belgium and, more generally, in Europe. It analyses the motivations and strategies of some Belgian actors engaged in advocacy for rare diseases since the early 2000s, before turning to the process of elaboration of an action program within the context of the European Project for Rare Diseases National Plans Development (2008-2015). Finally, the research addresses the implementation of the plan nowadays.

- The **qualitative methodology** is based on various tools:
  - Interviews with actors from the field of rare diseases
  - Participant observations at meetings and events
  - Documentary analysis

Findings

- Analysing the discourses and practices of the actors and communities involved in **the design and management of an action program for rare diseases in Belgium**, the research highlights **some initial points of agreement and tension**, such as the degree of adequacy between international guidelines and national constraints, the confrontation between professional and experiential points of view, or the multiple meanings and uses of the "rare diseases" category.

- Then, the research particularly focuses on the tensions between **the multiple meanings and uses of the "rare diseases" category**, as well as the ways in which it has locally been defined and mobilised by the diverse stakeholders. It finds that, on the one hand, at an individual level, **stakeholders' specific visions and claims** are identified regarding their different statutes, roles, and activities within the Belgian context. On the other hand, at a collective level, **some tensions underlying cooperation attempts** to address rare diseases issues may be pointed out:



- **In the process of elaboration of an action program…**

1 - The constitution of **the Fund for Rare Diseases & Orphan Drugs** (2009) as a "**trading zone"** (Galison, 1997) allowed gathering all stakeholders, managing communication despite the divergent points of view, and defining an homogenous "rare diseases" category.

2 - The publication of **the Belgian Plan for Rare Diseases** (2013) was a **successful outcome of the cooperation** between all stakeholders within the trading zone (Collins, Evans & Gorman, 2010).

- **In the dynamics of action…**

3 - The discrepancies between their statutes within the Belgian healthcare system render difficult **the maintenance of all stakeholders' representation** in the monitoring of the Plan for Rare Diseases, especially on the side of patients and relatives' associations.

3 - The constant (re)emergence of diseases and patients *as singularities*, which is inherent to the approach of rare diseases *as a whole*, weakens **the stakeholders' representativeness and the making of social links** aligning to the "rare diseases" category.

4 - Finally, the question remains open of **the evolution of the trading zone formed around rare diseases issues** at national and/or international level, that is, whether it is likely to consolidate, even to institutionalize, or to fade away? A comparison between different national contexts would be insightful on this point.

Conclusion

- **The "rare diseases" category** was initially far from self-evident; it has gone through a history that spans three decades and expanded worldwide, being progressively endowed with **the specificities that define its local meanings, uses, and relevance** today. Along this rich history, some actors have identified themselves in relation to rare diseases, and conversely, the combination of and coordination between their different needs, interests, and expectations defined **the plural and multifaceted – social, political, economic, medical, experiential, or moral – form of the category**.

- Beyond the presentation of findings from a fundamental academic research, this poster aspires to show the ways in which **a social science perspective is particularly valuable** for the rare disease community. Indeed, **multidisciplinary perspectives**, as well as **mutual exchanges between social scientists and stakeholders** will only be benefiting the joint understanding of, and cooperation in, the lively field of rare diseases.