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Solidarity in Health Research Regulation

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5.1 INTRODUCTION

This chapter explores the analytical and normative roles that solidarity can play when designing health research regulation (HRR) regimes. It provides an introduction to the meanings and practical applications of solidarity, followed by a description of the role solidarity plays in HRR, especially in fostering practices of mutual support between patient organisations and between countries. We illustrate our argument in a case study of HRR, namely the European Union (EU) regulatory regime for research on rare diseases and orphan drugs. The current regime aims to decrease barriers to research on orphan drugs by creating, predominantly financial, incentives for research institutions to take on the perceived increased risks in this area. We show how the concept of solidarity can be used to reframe the purpose of regulation of research on orphan drugs from a market failure problem to a societal challenge in which the nature of barriers is not just financial. This has specific implications for the types of policy instruments chosen to address the problem. Solidarity can be used to highlight the political, social, economic and research value of supporting research on rare diseases and orphan drugs.

5.2 THE MEANING OF SOLIDARITY

The concept of solidarity underpins many social and healthcare systems in Europe.[1] While it could be argued that solidarity – in the form of policies and institutional structures facilitating mutual support, with special emphasis on supporting the vulnerable – has come under pressure with the spread of nativist and other sectarian political ideologies, there are also forceful counter-movements under way. These include people standing up with and for others,[2] may it be newcomers to our society, victims of wars and natural disasters or people who suffer from our economic and political system. As such, it is fair to say that solidarity is seen by many as having a lot to offer to how we frame and address societal challenges.

[1] K. Kieslich, 'Social Values and Health Priority Setting in Germany', (2012) *Journal of Health Organization and Management*, 26(3), 374–383; L. D. Brown and D. P. Chinitz, 'Saltman on Solidarity', (2015) *Israel Journal of Health Policy Research*, 4(27), 1–5; R. Saltman, 'Health Sector Solidarity: A Core European Value but with Broadly Varying Content', (2015) *Israel Journal of Health Policy Research*, 4(5), 1–7; R. ter Meulen, *Solidarity and Justice in Health and Social Care in Europe*, (Springer, 2001).

[2] A. Dawson and B. Jennings, 'The Place of Solidarity in Public Health Ethics', (2012) *Public Health Reviews*, 34(1), 65–79.

What is solidarity? At first sight, it might seem an elusive concept. For decades, solidarity has been used to justify a wide variety of policies and practices ranging from vaccination programmes to biobanks to the penalisation of undesirable behaviours. Another reason for the elusiveness of solidarity lies in the practical and embodied nature of solidarity. Solidarity is, first and foremost, a relational practice: its full meaning unfolds only when it is enacted, in concrete practice, by – at least one – giver and a receiver, and its nature cannot be exhaustively captured by language. For the same reason that poetry, art or nature are so much more powerful in conveying the meaning of love or friendship, words alone struggle to convey the full meaning of solidarity.

Acknowledging that part of the meaning of solidarity resides in its embodied- and enactedness does not mean, however, that we cannot spell out what makes solidarity different from other types of prosocial practice. Building upon a long history of scholarship on solidarity we have, in our own work, proposed that solidarity is best understood as a practice that reflects a person's – or persons' – commitments to support others with whom the person(s) recognise(s) similarity in a relevant respect.[3] The similarities with others that people recognise are, however, not 'objectively' existing properties, but they are characteristics that we have learned to attribute to ourselves and to others. The first step in this process is that we use categories that have been developed to sort people in different groups, such as separating them into women and men, children and adults, Jews, Buddhists and Muslims, or Koreans and Croatians. While these categories clearly have an expression in material reality, such as the correspondence of national labels with specific territories, or – in the case of children and adults, even stages in human biology – these categories are not merely material. To whom the label of 'Korean' or 'Croatian' is applied has not been stable in history but it has depended on changing territorial rule, changing understandings of nationality and different perspectives on who can legitimately claim belonging to such a label. Similarly, the notions of children and adults are not clearly delineated in biology in the sense that every person neatly fits into one or the other category. In this way, the categories that we use to describe characteristics that we and others hold are lenses through which we have learned to see reality.

For solidarity this means that when a woman supports another person because she recognises her as a fellow woman, then 'being a woman' is the 'similarity in a relevant respect' that gives rise to solidaristic action – despite the fact that the two people in question are many more things than women. They may be different in almost every other way. In this sense, the recognition of similarities in a relevant respect is a subjective process – I recognise something in you that you may not recognise in yourself because you have not learned to see it. At the same time it concerns shared social meaning – as societies have shared conventions about how they classify people.

Solidarity happens when people are guided in their practices by the similarities they recognise with each other, despite everything that sets them apart. It is the similarities, and not the differences, that give rise to action in the sense that they prompt people to do something to support somebody else. This 'doing something' could consist of something big – such as donating an organ – or something small, such as offering somebody a seat on a bus.

In sum, what makes solidarity different from other pro-social practice is the symmetry between people in the moment of enacting solidarity. This symmetry is not an essentialist ontological statement that glosses over claimed or ascribed differences and structural inequalities. Instead, it

[3] B. Prainsack and A. Buyx, 'Solidarity: Reflections on an Emerging Concept in Bioethics', (Nuffield Council on Bioethics, 2011); B. Prainsack and A. Buyx, *Solidarity in Biomedicine and Beyond* (Cambridge University Press, 2017).

is the description of a relational state in the moment of enacting solidarity. In this way, solidarity is distinct from other pro-social supportive behaviours such as cooperation and charity, for example. The notion of cooperation describes pro-social supportive behaviour without saying anything about how and why people engage in it. The notion of charity describes an asymmetrical interaction between a stronger entity giving something and a weaker entity receiving something. In contrast, solidarity refers to entities that are different in many respects but make the thing they share in common the feature upon which they act: I do something for you because I recognise you as a fellow woman, a co-worker who struggles to make ends meet, as I do, or a fellow human in need of help.

5.3 THE THREE TIERS OF SOLIDARITY: APPLICABILITY AND ADJUSTMENTS IN THE CONTEXT OF HEALTH RESEARCH REGULATION

Having defined solidarity as practices that reflect commitments to support others with whom a person – or persons – recognise(s) similarities in a relevant respect, in previous work one of us identified three main tiers of solidarity, capturing the societal levels where solidaristic practice takes place.[4] Tier 1 is the interpersonal level where solidarity is practised between two or more people without that practice having become more widespread. An example from the field of health research would be a person with diabetes signing up to a biobank researching the disease because she wants to support others with similar health problems.

If this practice were to become more widespread, so that it became common or even normal behaviour within a group, then we speak of solidarity at Tier 2 solidarity, which is solidarity at the group level. The group within which solidarity is practised could be a pre-existing group – such as a self-help group around diabetes where it becomes normal practice, for example, to also volunteer for disease research – or a group that is created through the solidaristic practice itself. An example for the latter would be a patients' rights organisation created in response to the effects of harmful medical practices such as the blood contamination scandal in the 1970s and 1980s in the United Kingdom (UK).

If solidaristic practices become so commonplace that they are reflected in legal, administrative or bureaucratic norms, then we speak of Tier 3 solidarity. This is the 'hardest' form of solidarity because it has coagulated into enforceable norms. Tier 3 solidarity could be seen to contradict the idea held by many scholars in the field that solidarity cannot be demanded, but only appealed to.[5] In this understanding, contractual and legal obligations are incompatible with solidarity. While we agree with these authors that solidarity is typically a more informal, voluntary 'glue' between the bricks of formal institutional arrangements, we also believe solidarity to be a toothless, if not empty, concept if it cannot also denote practices that are so deeply engrained in society that they become legally enforceable in some cases.

Ruud ter Meulen and colleagues very helpfully distinguish between solidarity as a community value and solidarity as a system value:[6] the latter can contain articulations of solidarity in formal, often legal arrangements. The key here is to consider enforceable – and thus not always voluntary – solidarity in conjunction with more informal, voluntary forms of solidarity, and not see them as isolated from one another. An example would be tax or contribution-based financing of universal healthcare where those with higher incomes contribute more than others.

[4] Prainsack and Buyx, 'Solidarity: Reflections'; Prainsack and Buyx, *Solidarity in Biomedicine and Beyond*.
[5] J. Dean, *Solidarity with Strangers: Feminism after Identity Politics* (Berkeley: University of California Press, 1996), p. 12;
[6] ter Meulen, *Solidary in Health and Social Care*, p. 11.

A problem arises when legally enforceable solidarity is still in place while the actual practices that used to underpin them are breaking away. This is becoming apparent at the moment in many countries where certain features of welfare states, such as transfer payments in the form of as child allowances or income support for those considered undeserving, have come under attack. The argument is often that the people benefitting from this are 'free riders' as they have not contributed towards the system that they are now using – perhaps because they are new immigrants or people who have never been in paid employment. What is happening here is that the basis for solidaristic practice – namely the 'recognition of similarity in a similar respect' (see above) – is breaking away. The people who are receiving financial support, or benefitting from a solidaristic healthcare system, are no longer seen as belonging to 'us' – because of something that they supposedly did, or failed to do, or because they do not have the same passport as we do.

While it will often be the case that solidarity prescribed at Tier 3, in the form of legal, contractual, bureaucratic and administrative norms, will have evolved out of solidarity practised at group (Tier 2) and interpersonal (Tier 1) levels, the reverse is not necessarily true: interpersonal solidarity can, but does not necessarily, scale upwards. The 'higher' the level of solidarity, the more important reciprocity becomes. Here we refer not to direct reciprocity, where one gives something in return for something else – this would be a business transaction instead of solidaristic practice – but indirect, systemic reciprocity. Institutional arrangements of solidarity work best when people give because they want to support others, but they also know that when they are in need they will be supported as well.

5.4 SOLIDARITY IN HEALTH RESEARCH REGULATION

How do the aforementioned conceptualisations of solidarity apply to HRR regimes? The first aspect we need to acknowledge is that HRR regimes are complex and varied. There is no such thing as one regime that applies to all areas of HRR, but rather there are multiple and sometimes overlapping legal and ethical requirements that need to be fulfilled by those planning, funding, supporting and undertaking research. HRR is a multidisciplinary endeavour that involves different actors such as policymakers, researchers, health professionals, industry and patients. HRR also spans a large variety of 'objects' that are regulated, such as data, tissue, embryos, devices or clinical trials.[7] This means that it occupies regulatory spaces beyond health, such as in data regulation, research financing, in fostering innovation and in the obligation to protect research recruitees.

At the start of this chapter we suggested that solidarity can be thought of as 'enacted commitments to accept costs to assist others with whom a person or persons recognise similarity in one relevant respect'.[8] Thus the question arises: what are the shared practices that reflect a commitment to carry costs – emotional, financial, societal – in HRR, and what are the similarities that give rise to these practices? The two tiers of solidarity most relevant in HRR are Tiers 2 and 3. Tier 2, or group solidarity, is reflected, for example, in the way patients, patient groups and other stakeholders advocate for, inform about, and partake in research endeavours and regulatory steps to make them happen. The question of who partakes in research is not just important for methodological reasons but is also connected to the concept of solidarity. It is considered good scientific practice to carry out research in the populations

[7] G. Laurie, 'Liminality and the Limits of Law in Health Research Regulation: What Are We Missing in the Spaces In-Between?', (2016) *Medical Law Review*, 25(1), 47–72.

[8] Prainsack and Buyx, *Solidarity in Biomedicine and Beyond*, p. 43.

for whom an intervention is intended, but there may be instances in which it is justified to conduct research in populations other than the intended beneficiaries. According to the Council for International Organizations of Medical Sciences (CIOMS) and the World Health Organization (WHO) such instances are 'important demonstration[s] of solidarity with burdened populations',[9] for example in 2014 when Ebola vaccines were tested in communities not affected by the Ebola outbreak.

The costs and the similarities that are at the heart of these – predominantly clinical – research practices are comparatively easy to identify. The costs commonly consist of individuals giving up their time to become research participants or to become involved in a patient advocacy group. They accept the burden of cumbersome regulatory steps to partake in research, such as navigating consent forms, risk assessments, data ownership and other issues. The similarity that motivates people to assist others despite the costs they incur is often the experience of suffering from a particular disease or the acknowledgement that we, as members of society or those close to us, all run the potential risk of illness in the future. It is a recognition that temporary sacrifices can result in long-term gains from the generation of new knowledge about health conditions and treatments.

A feature that distinguishes HRR from other areas of policy, regulatory and societal processes is that group solidarity is often not just confined to a small group of patients who are afflicted by the same illness. Rather, other members of the public – so-called healthy recruits – partake in the solidaristic practice of research and are directly affected by the associated regulatory procedures. The underlying 'similarity in a relevant respect' that, in Prainsack and Buyx's definition of solidarity gives rise to solidaristic practice, is then typically a broad sense of human vulnerability that we all have in common. In other words, the nature of Tier 2 solidarity in HRR is not necessarily restricted to suffering from the same illness, but it can arise from the recognition that in a universally funded healthcare system, we all carry a commitment to carry costs because we all carry the risk that we might one day become ill.

To explore how Tier 3 solidarity, or institutional solidarity, is reflected in HRR, we trace the logic that forms the basis for understanding HRR through the lens of solidarity. The logic runs something like this: A solidaristically financed healthcare system is built on the principles of fair access to healthcare, protection against financial risks due to illness and quality. Ensuring access, provision and high-quality healthcare requires efforts to advance knowledge through research. Implicitly entailed in the social contract between governments, citizens and residents is the acceptance that mandatory financial contributions – i.e. costs – in the form of taxes or health insurance contributions will not only be used for the day-to-day provision of services but also for the fostering of research activities. With this implicit acceptance of carrying costs collectively comes a recognition that the health research area needs to be regulated to safeguard against unethical, harmful, and wasteful practices, and to foster innovation. This recognition translates into public policies that regulate the field.

But there are also regulatory burdens arising from such public policies that might negatively affect solidarisic practices in HRR. For example, the cumbersome, and often time-intensive, process of giving consent for a research participant's data to be used for research purposes might deter some people from taking part in a study, especially if the use of the data is not explained or communicated clearly. Moreover, the predominant lens through which data ownership – in a moral and in a legal sense – is currently viewed is that of the rights of individuals, who, in turn,

[9] Council for International Organizations of Medical Sciences, and World Health Organization, 'International Ethical Guidelines for Health-related Research Involving Humans', (CIOMS, 2016).

are conceptualised as bounded and independent entities.[10] This view is problematic because it fails to acknowledge the deeply engrained relational characteristics of data. This is so because the meaning of most data only unfolds once the data is interpreted in relation to other data, and that this meaning is often relevant for a wider range of people than only the person from whom they came. Currently, this relational nature of data is not reflected in most data governance frameworks in the health domain; even those frameworks that give people more control over how their data is used typically give this control to individuals. Instruments of collective control and shared ownership of personal data are rare. The 'individualisation' of data governance sits squarely within a system that relies on people's willingness to make data about themselves available for research. It is a missed opportunity for showing how control and use of data can reflect both personal and collective interests and rights.

5.5 SOLIDARITY IN RESEARCH ON RARE DISEASES AND ORPHAN DRUGS

An example of how solidarity can be used to change the way we approach a policy problem in HRR can be found in rare diseases and orphan drugs research. The European Commission (EC) defines a rare disease as 'any disease affecting fewer than 5 people in 10,000 in the EU'.[11] It estimates that there are approximately 5,000–8,000 rare diseases in the world. The challenge around rare diseases is that the comparatively small numbers of people affected by them translate into the neglect or the unavailability of diagnoses and treatment options. It can be explained by drawing on the notion of issue characteristics, famously developed by political scientist Theodore Lowi.[12] Lowi posited that different types of policies – e.g. regulatory, distributive or redistributive policies – give rise to different policymaking or decision-making processes through which distinct patterns of political and societal relationships and behaviours emerge. Just as the categories we use to describe characteristics that we hold – women and men, adults and children, Koreans or Croatians – we can use categories to describe characteristics that policies or policy fields hold. For example, the depiction of European healthcare and welfare systems as solidaristic has arisen from their embeddedness in redistributive policies that allow the state to redistribute taxes and other welfare contributions in the pursuit of policy goals. Different types of policies give rise to different forms of state action, but also to different types of public participation, or even political controversy and contestation. The latter is what we frequently observe when a change in redistributive policies is suggested. Following Lowi's rationale, the key to understanding patterns of behaviours, in this case the lack of attention given to rare diseases, is to identify the characteristics of the issues to which they give rise. The more complex the regulatory or policy area, the more difficult it is to develop policy solutions.

The issue characteristics for rare diseases are complex. We know relatively little about the factors and processes that underlie these diseases. This stems from a lack of basic research into rare diseases[13] which is mostly due to a lack of available funding for research that a relatively small number of people suffer from. From a public policy perspective, the question of how and if to prioritise research for rare diseases is an intrinsically complex issue because of the low

[10] B. Prainsack, 'Research for Personalised Medicine: Time for Solidarity', (2017) *Medicine and Law*, 36(1), 87–98.

[11] European Commission, 'Rare Diseases', (European Commission, 2018), www.ec.europa.eu/health/non_communicable_diseases/rare_diseases_en

[12] T. J. Lowi, 'American Business, Public Policy, Case-Studies and Political Theory', (1964) *World Politics*, 16(4), 677–715.

[13] EURORDIS-Rare Diseases Europe, 'EURORDIS' Position on Rare Disease Research', (EURORDIS, 2010), www.eurordis.org/sites/default/files/EURORDIS_Rapport_Research_2012.pdf

numbers of patients and the high costs for research and treatment. It begs the (redistributive) policy question how spending a large proportion of overall research or healthcare budgets on a few patients can be justified if the opportunity costs are such that other patients may lose out as a result. The low patient numbers also result in difficulties in the design of clinical trials that meet the evidentiary hurdles of most regulatory agencies in Europe.[14]

Solidarity offers a lens through which these difficult questions surrounding research on rare diseases can be reframed. Patients suffering from rare diseases are characteristically vulnerable (please see Rogers' Chapter 1 in this volume for more detail on the concept of vulnerability). Their vulnerability results from the severity and the chronicity of their conditions, the inadequate access to appropriate diagnoses and treatment options, societal isolation and a lack of representation of their interests.[15] Coming back to the importance of Tier 3 solidarity in HRR (the institutional and legal level), the solidaristic principles upon which healthcare systems in Europe rest suggest a duty to care for society's most vulnerable members, which patients with rare diseases undoubtedly are. Policies or regulations to support research and service provision for patients with rare diseases can therefore be viewed as solidaristic practices.

However, despite initiatives such as the introduction of Regulation (EC) 141/2000 on orphan medical products, access to adequate services and research for patients is still falling short of expectations. Following Lowi's approach, as outlined above, we can observe that the more complicated the issues to which a regulatory or policy area give rise, the less policymakers are inclined to act because of the perceived lack of policy options. This might also explain why the challenges around fostering research activity on rare diseases are predominantly framed as a regulatory policy problem rather than a distributive or redistributive one. Interestingly, the perceived lack of policy options and responses corresponds with a flourishing of solidaristic practices below the level of public policy that span borders and countries at the EU level. For example, there seems to be an emerging recognition of 'similarity in a relevant respect' among EU countries in the sense that the issue characteristics of rare diseases are such that no country can stem the challenge of protecting vulnerable patients suffering from rare diseases on its own. Here, Tier 2 solidarity does not just apply to the level of interaction and collaboration among patient groups, but also to the level of cooperation between nation states. The similarity is the recognition that all countries face the same challenge in finding adequate research and treatments on rare diseases – the policy problem – and that countries are similar in their failure to find policy solutions. This can lead to the fostering of solidaristic practices such as the EC's advocacy for a European Platform on Rare Diseases Registration that would bring together patient registries and databases to encourage and simplify clinical research in the area.

An unresolved question in the application of a solidarity-based approach to the field of HRR is the role of industry, especially in fostering or hindering solidaristic practices. It is frequently argued that pharmaceutical manufacturers do not invest enough resources into the research and development of rare diseases and orphan drugs because the small patient numbers lead to a low return on investment (RoI).[16] The response of EU member states has been to create incentives through policy instruments such as fee waivers for regulatory procedures or a 10-year market exclusivity for authorised products.[17] The introduction of such measures in the Regulation (EC) 141/2000 on orphan medical products has increased the number of orphan drugs being

[14] Ibid.
[15] Ibid.
[16] E.g. ibid.
[17] European Commission, 'Rare Diseases'.

authorised. But is it also a sign that pharmaceutical industries are engaging in solidaristic practices to benefit some of the most vulnerable patients?

We argue that it is not. We must assume that pharmaceutical companies are motivated by the incentives offered through this regulation rather than a recognition of similarity with entities that seek to promote public benefit, or with people suffering from illness. The perception that some people, as taxpayers or patients, are expected to contribute to supporting others who suffer from rare diseases, while some corporate actors do the bare minimum required by law, may have a significant negative effect on the people of other actors to contribute. This may be exacerbated by the payment by corporations of hefty dividends to their shareholders. Institutionalised solidarity requires some level of reciprocity – the understanding that each actor makes a contribution adequate to their nature and ability. As a result, if large multinational companies are seen to get away with 'picking the raisins' this is a serious impediment to solidarity.

In a field that is still very dependent on the investment of pharmaceutical companies into drug research, resolving this challenge of asymmetry is not easy to rectify in the short term. Its solution would require legislation that forces companies to cut their profits and support rare disease patients in more significant ways than they are doing at present. A for-profit company cannot reasonably be expected to be motivated by the desire to help people; it is to be expected, and justified, that they put profits first. This is why it is the role and responsibility of legislators to ensure that companies are contributing their fair share. This is not only a necessity for moral and ethical reasons, but also to avoid the hollowing out of solidaristic practices among people who may, as argued above, be deterred by the expectation to accept costs to help others, while others are making huge profits.

The concept of solidarity can and should be used to reframe the regulation of research on orphan drugs from a market failure problem that requires financial incentives, to a societal problem that requires more than market measures. This will require a reframing of the issue as a redistributive policy problem rather than a purely regulatory one, in the hope that this will instigate political debates, as well as patient and public participation that would help bring the challenges of research on rare diseases and orphan diseases more to the centre of the policy process. Using the concept of solidarity to help reframe the policy issue has the potential to draw it out of the comparatively confined policy spaces it currently occupies. This helps to illuminate its political and public salience. The joined-up working of patient groups for rare diseases and the mutual efforts of EU member states – also as regulators that impose rules of fair play on pharmaceutical companies – are needed to facilitate – and where they already exist, stabilise – solidaristic practices. To make these practices more powerful and meaningful, priority-setting mechanisms for the prioritisation of research funding need to be developed,[18] and more public money should be invested, especially into basic research, in an effort to decrease the dependence on the pharmaceutical industry.

5.6 CONCLUSION

In this chapter, we have used research on rare diseases and orphan drugs to highlight the application of solidarity to HRR. It is an example of a space where solidaristic practices are already taking place, but also illustrates that there is room for improvement. Solidarity is an integral part of health research, and it is enacted every time a person takes part in a clinical trial

[18] C. Gericke et al., 'Ethical Issues in Funding Orphan Drug Research and Development', (2005) *Journal of Medical Ethics*, 31(3), 164–168.

or other research because they want to support the creation of public benefits. Regulation is important to ensure that research is carried out in an ethical manner, but, equally, it is important that decision-makers who define the regulatory spaces for HRR recognise the need to support solidaristic practices rather than undermine them through overly cumbersome bureaucratic hurdles to enrol in research.

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