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Patient advocacy and articulation of expectations about pharmaceutical innovations

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Abstract

Objective: to show how patient advocacy organizations articulate and manage expectations about pharmaceutical innovations when faced with issues pertaining to unsustainable drug development, such as unequal access to new medicines, debatable medical need, or low economic profitability.

Background: the management of expectations by patient advocacy organizations is increasingly gaining importance in influencing pharmaceutical innovations and can have a positive influence on keeping health care sustainable.

Methods: concise stylized examples in the field of pharmaceutical innovations were drafted based on in-depth research into the patient organizations. This research conveyed a large amount of events in which these organizations were engaged using a triangulation of methods and sources (archival materials, interviews with representatives of patient organizations and other actors in the health care system, observations of meetings and conferences).

Findings: patient advocacy groups are to a large extent engaged in new technologies. The most important ways of managing expectations are 1) achieving a balance between making use of enthusiasm and the dynamics of expectations and ‘forced forwarding’ or overemphasizing risks, and 2) forming and communicating expectations and visions in the context of societal debates.

Conclusions: the variability of patient advocacy groups and their contexts leads to broadening and enriching the debates on the sustainability of drug innovation and to differences in attention to certain sustainability issues, including ethical and social impacts, access, and solidarity. The heterogeneity, subtlety, diversity and variability of patient advocacy make these organizations complex and underestimated vehicles for the articulation of expectations.

Keywords: pharmaceutical innovations, patient advocacy, expectations, sustainability.

Patient advocacy and articulation of expectations about pharmaceutical innovations

1. Introduction

The health care industry, consisting of a diverse range of companies and related products, such as pharmaceutical products, diagnostics, and medical appliances, is traditionally an innovative one. Recently, scholars have questioned the innovative capacity of pharmaceutical companies. They based their discussions on moral arguments or on macro-trend figures, showing the existence of an ‘innovation deficit’.¹ These arguments claim there is too much emphasis on so-called ‘me-too’¹ and generic drugs,² and even on promoting pharmaceutical solutions to mild ailments. There is less focus on meeting medical needs, such as drugs for complex, rare, or neglected diseases.²

Rising drug development costs inevitably lead to higher prices for drugs, which in turn form a heavy burden on public and private health care expenditure. Already, pharmaceutical products constitute an increasing portion of these costs.⁴ This can be problematic when pined against still unmet medical needs, like cures for Parkinson’s disease or malaria.⁵ Because of the growing investments, pharmaceutical companies try to reduce risks and do not prefer investing in non-established, new technologies, such as pharmacogenetics.⁶ These technologies entail great expectations and promises, although at the same time their characteristics are uncertain and less concrete.^{7,8}

These problems faced by the pharmaceutical industry relate to the concept of *sustainability of drug innovation*, which encompasses the extent to which present needs in the health care system in general can be met for current and future generations. This poses questions about whether pharmaceutical innovations show safety and efficacy and deliver sufficient value for money; whether they are accessible on acceptable ethical grounds; and whether the benefits and costs are equally distributed.

Accordingly, the healthcare and pharmaceutical sector faces several problems, some of which are sketched above. In this paper, we focus on three important issues: 1) unequal access to medicines, 2) debatable medical need (disease mongering), and 3) low economic profitability (orphanization). Dealing with these problems, and sustainable drug development in general, involves adopting a long-term perspective on drug innovations, taking into account the inherent uncertainties and flexibilities involved. In this context, the *articulation and management of expectations* about new technologies plays an important role. In order to turn this management into a sustainable exercise, a wide range of commentators regard the engagement of the public, care providers, and patients in these discussions as invaluable. A special group of stakeholders that is increasingly gaining importance in health care is *patient organizations*. In the context of drug sustainability, patient organizations are the main problem owner because they have specific preferences related to medical needs, access to medicines, and so on. In recent decades they have broadened their efforts including not only mutual help, support, and communication to their patient members, but also advocacy activities. The articulation of

¹ Me-too drugs are similar to and only marginally different from medicines that have already been on the market for some time.

² This deficit was criticized as well.³

demands and the management of expectations by patient organizations might illuminate their way of dealing with the sketched unsustainable situation, and might benefit the shaping of new technologies and their related innovation processes. These benefits can take several forms, such as making use of users' creative potential and experiential knowledge,⁹ enlarging the democratic quality of debates, and recruiting support for decisions, but also resolving discussions between actors with different norms and knowledge systems¹⁰ and dealing with potentially inflated expectations and requirements of new technologies.¹¹

The *objective* of this article is to show how patient advocacy organizations articulate and manage expectations about pharmaceutical innovations when faced with issues pertaining to unsustainable drug development, such as unequal access to new medicines, debatable medical need, or low economic profitability.

In the following two sections the role and importance of expectations and patient advocacy organizations are treated. The conceptualization of the management of expectations is illustrated by stylized examples of patient organizations dealing with the three major sustainability issues in drug innovation as mentioned above. In the last section we discuss the extent to which these results provide insight into the management of expectations by patient groups in the context of drug sustainability problems.

2. Conceptualizing expectations about innovations in health care

A wide range of stakeholders, like scientists, medical specialists and patient groups, constantly and actively shape new and emerging technologies. In the process of doing so, they are confronted with and articulate representations of future technological situations and expectations. This involves the articulation of expectations and opinions regarding different aspects of the technology, including the supporting infrastructure, the inclusion of actors, target groups, and of course specifications of the technology itself, based on their preferences or results of scientific research. Ideally, articulation entails both gaining more depth (specification) and choosing between alternatives (directions). Figure 1 presents this articulation (a).

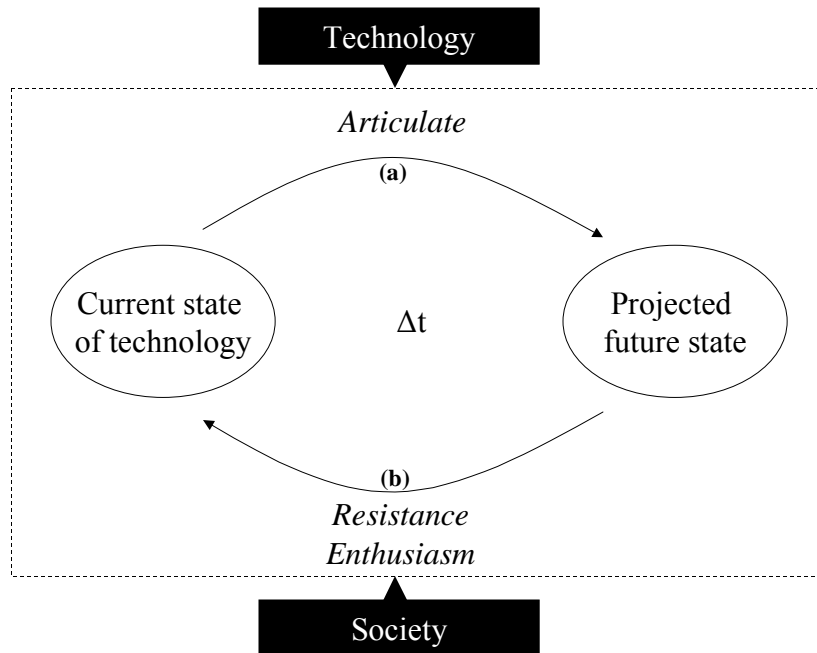


Figure 1: Expectations about technologies projected and articulated into the future and the perceived consequences for the present.

Subsequently, this projection of a new technology results in *perceived consequences* for society that have an impact on the present way of thinking about this technology. There are two groups of consequences that can be discerned, namely consequences related to enthusiasm and to resistance (b). Both groups of consequences can have positive and negative impacts.

Enthusiasm

Projections of new technologies prompt stakeholders to interpret these innovations and value their merits. In a sense these interpretations are an integral part of the projection itself. Positive elements of these potential innovations are emphasized and include promises to solve problems (new safe and efficacious medicines to cure a disease), move to new frontiers (tissue engineering, individualized medicine), and also connect to societal and cultural movements that are simultaneously occurring (oral contraceptive pill in the 1960s).

These promises lead to enthusiasm in involved stakeholders. This enthusiasm has positive and negative impacts in itself. A positive effect is that a technology's increasing visibility and positive annotation result in it entering the local and shared agendas of stakeholders, including initially less involved ones. The shared character and careful scrutiny under which these actors are acting cause expectations and promises to be translated into requirements. Therefore, expectations are regarded as 'performative'¹² and in this way, stakeholders' engagements and mutual dependencies might result in higher investments. The negative impacts concern, firstly, negative dynamics when the expectations and promises are not fulfilled (within a reasonable time frame).

Disillusionment and disappointment might even result in a negative image that sticks to the technology and damage proponents' credibility.¹³ Secondly, stakeholders might be engaged in the promises of a new technology to such an extent that they seize the opportunity to accelerate the developments or even move the technology forward in time ('forced forwarding'). The motives of some stakeholders, such as patients, are straightforward, especially if they are terminally ill. This is illustrated by the case of the potential cancer drug dichloro-acetate (DCA). This compound had been through preclinical trials and proved to be successful in stopping tumour growth in rats. Recently, a company set up by a cancer patient's relative started to produce and sell the drug although it has not yet been approved for use in humans.¹⁴ Also in the case of stem cell therapy, despite the fact that possible therapeutic products are not expected for the first ten years, some firms sell therapeutic and biobank services based on this promise.¹⁵ These companies and users jeopardize not only patients' lives but also the (image of the) entire new technology.

Resistance

The projected new technology might also have perceived negative impacts, for example concerning the safety (risks), efficacy, or implementation process (reimbursement, regulation) of the product. Also, a mismatch can occur between the developments and the prevailing cultural norms. This leads to fear, anxiety, and even resistance.

This resistance again can have positive and negative consequences. Negative consequences are related to actors who are taking an anti-technology or anti-innovation stance.

Table 1 summarizes the potential positive and negative consequences of new technologies for stakeholders in innovation processes.³

³ Despite the presentation of the table, 'enthusiasm' and 'resistance' should not be regarded as two mutually exclusive categories.

Table 1: Summary of consequences of reactions to new technologies.

| Groups of consequences | Consequences | Impacts of consequences | |
|------------------------|--|--|---|
| | | Positive | Negative |
| Enthusiasm | Promises to solve problems | Increasing visibility and positive annotation resulting in engagement and requirement for action | Disappointment resulting in loss of credibility 'Forced forwarding' and related risking of entire technology |
| | Move to new frontiers | | |
| | Connect to societal and cultural movements | | |
| Resistance | Perceived negative direct, indirect, and/or unintended impacts, for example concerning the safety (risks), efficacy, or the organization of the product's implementation (reimbursement, regulation) | All stakeholders are heard, which has instrumental, moral, and political advantages | Anti-technology or anti-innovation movements |
| | Mismatch can occur between the developments and the cultural norms | | |

3. Patient advocacy and managing expectations

The world of patient advocacy organizations is heterogeneous in several dimensions, such as objectives and ambitions, roles, cultures, and scope. There are several stages in the drug R&D chain where patient groups (increasingly) try to be influential. Figure 2 illustrates the drug innovation process as a nonlinear model, which includes feedback and feedforward loops, cyclic interactions inside companies, and interactions with research partners.¹⁶ Patient advocacy organizations play different roles in these processes, several of which pertain to the articulation and management of expectations.

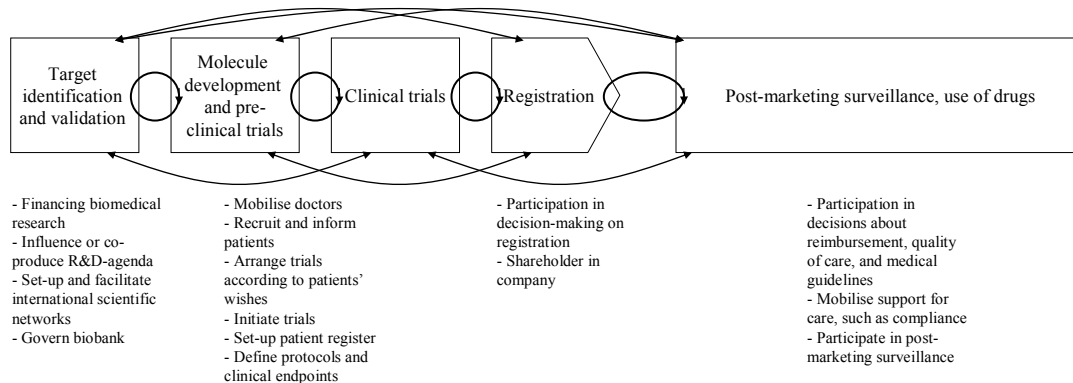


Figure 2: Non-linear model of the drug innovation process and the ways in which patient organizations can influence this process.

Patient groups have become leading players in funding and steering biomedical and clinical research.¹⁷ Prominent examples in the literature of patient groups involved in research are the Alzheimer’s Society in the UK, the French Muscular Dystrophy Association,¹⁸ and the Netherlands Asthma Foundation.¹⁹ While co-producing biomedical research agendas, these organizations engage in interactions with scientists, which ensures that the information they have is up-to-date and the expectations are more balanced. The articulation of expectations potentially promotes scientific developments.

In a later stage, patient groups play a crucial role in organizing clinical trials, for example by mobilizing patients, medical specialists, and funding and co-defining protocols and clinical endpoints. A notable study by Epstein²⁰ focused on AIDS clinical trials. AIDS activist groups have been “*mobilized to develop effective social movement organizations that evaluate knowledge claims, disseminate information, and insert laypeople within the process of knowledge construction*”. They have done this by presenting themselves “*as credible speakers about science, and did so, in part, in the language of biomedicine*”. They even transformed “*the very definition of what counts as credibility*”. Later, breast cancer activists followed these efforts while advocating breast cancer treatment.²¹ This has been supported by research on patient groups in the UK¹⁷: two case studies illustrate how they mobilized epistemic groups, defined trial protocols, leveraged public money, brought forward experiential knowledge, and recruited patients. In this clinical trial stage, expectations become more articulated and encourage patient groups to take actions, for example by supporting boundary conditions for clinical trials.

Lastly, advocacy activities in patient groups focus on the later stages of the drug innovation process: the market phase. Prominent examples are the inclusion of patients as witnesses before technology appraisal panels in reimbursement decisions made by the National Institute for Clinical Excellence in the UK,¹⁷ and influencing the availability of medicines by putting pressure on regulatory and reimbursement bodies, quality improvements of care processes, health insurance companies, education, transport, and labour conditions.²² Also in public debates on ethical, legal, and social impacts of

technologies and the related societal acceptance or resistance, patient advocacy groups can play a role as ‘champions’ among public groups who stress the benefits of a technology in reply to those that use a ‘rhetoric of fear’.^{23,4} A lot of these debates deal with the impacts of emerging science and technology, in which expectations play a large role.

4. Methodology

The way in which patient organizations articulate and manage expectations about the different types of drug sustainability problems, as introduced in the first section, will be illustrated by presenting concise stylized examples. By choosing prominent and striking cases we hope to counterbalance the fact that these examples cannot be regarded as representative for all patient groups. For every problem one patient group was investigated in depth. Because of comparability we focused on Dutch patient organizations, which were subsequently selected based on two criteria. Firstly, the organization should be regarded as a striking example with respect to its particular drug sustainability problem. This was the result of a round of interviews with experts coming from different corners of the Dutch health care system. Secondly, the organizations should be explicitly following or influencing pharmaceutical innovations as one of their major objectives. The organizations that are presented are complemented with prominent examples coming from patient advocacy literature or are examples mentioned while doing the research.

These stylized examples themselves are the result of in-depth research into the patient organizations involved. Firstly, identifying the major activities of the patient organizations during a quick scan, using introductory interviews with the organizations’ representatives, and reading (annual) reports of the organizations led to discerning interesting topics related to emerging technologies. Secondly, data were obtained from the organizations’ archives, using sources such as minutes of (board and committee) meetings, letters, reports, and evaluations. Other information sources include more open-access data, such as the organizations’ public websites. In addition, we conducted interviews with several representatives of these intermediaries ranging from chairmen and managing directors to researchers and secretaries. In some cases observation methods were also applied by being present during (invitational) conferences and meetings organized by the intermediaries. Thirdly, the organizations with which these patient groups interacted were investigated in the same way, albeit somewhat less in depth, by means of interviews. Lastly, these data were transformed into a narrative based on which we draw the presented results. This narrative was presented to representatives of the patient organization, their interacting partners, and more independent experts in order to obtain verification of the results found.

The activities in which all patient groups studied were engaged concerning pharmaceutical innovations (totalling 4014) were recorded, making use of a triangulation of methods and sources: 938 archival materials and 28 interviews with representatives of these organizations and those who interact with them.

⁴ It should be stressed that not all patient groups are unconditionally in favour of innovations. Besides that, the influence of patient organizations varies because of differences in professionalism, the degree to which they are taken seriously (or seen as mere ‘window dressing’), the quality of representation,²⁴ transparency, and independency of the pharmaceutical industry.²⁵

5. Illustrative cases of the articulation and management of expectations

I. Unequal access to medicines: the case of Herceptin⁵

Pharmaceutical innovations that answer to economic viability, technological feasibility, and medical needs are expected to have their own drivers for development. In this case it is hard to imagine a role for patient groups in the early stages of R&D. Nevertheless, when the drugs enter the market, they might be influential. A recent case revolves around the reimbursement of the expensive breast cancer drug trastuzumab (Herceptin). In the Netherlands, the reimbursement of expensive drugs that are used in hospitals has been an issue for over ten years, since the introduction of taxanes. The debate took a new turn when the first results of a large clinical trial on the efficacy of Herceptin in early-onset breast cancer were presented in 2005. The Dutch society of oncologists decided to include the drug in their medical guidelines of good practice. However, Herceptin had not been approved for early-onset treatment by then, so the drug could not be reimbursed for this indication. Hospitals were presented with a problem: their medical specialists needed to adhere to the good medical practice guidelines, while insurance companies were not compelled to finance these treatments. Moreover, for expensive intramural drugs in general up to 75% of the costs were backed by insurance companies, leaving a heavy pressure on the budgets of hospitals that treat lot of expensive drug users.

The looming problem of patients not getting optimal treatment induced the Dutch Breast Cancer Association (BVN) to organize support for ensuring Herceptin treatment and reimbursement and for a more durable solution regarding reimbursing expensive intramural drugs. The BVN did this by enlisting a heterogeneous group of organizations ranging from hospital representatives to the media, pressurizing the health ministry to come up with an acceptable solution. The BVN used arguments like solidarity (the right to be treated), good medical practice, ‘postcode lottery’ treatment (demonstrated regional differences in treatment), and the expectation of a future increase in expensive biotechnology products.

In the UK a similar discussion was going on, also featuring looming regional differences in Herceptin treatment and reimbursement of early-onset use. Patient groups, such as Fighting for Herceptin, and individual patients attracted a lot of attention in the media. Critics claim that the UK National Institute for Health and Clinical Excellence (NICE) was pressured into admitting Herceptin to be reimbursed far too quickly.²⁷ The UK approach differed from the Dutch one regarding the evidence for inequality that was presented (macro-figures versus individual stories) and the extent to which activist rhetorics were used.

This example shows that patient organizations that deal with unequal access to medicines are engaged in boundary conditions and are consciously striving after their rights, for example, for access to drugs. They typically focus on later-stage innovation

⁵ In the light of the case of genomics (see example II), we are aware that some authors regard the drug as one of the first successful examples of pharmacogenomics, although others dispute this by claiming that Herceptin only works on overexpression of proteins and genes in (somatic) tumour cells.²⁶ Apart from this scientific contestation, industry watchers claim that Herceptin is an interesting precursor for the pharmacogenomics future but the fulfilment of the advent and promises of the pharmacogenomics future could still be fifteen to twenty years away.

issues, like reimbursement. At the same time, some patient groups are aware of larger technological, societal, and cultural movements, such as the imminent increase in expensive (biotechnology) drugs, and discussions on the balance between private versus public drug reimbursement. In this way, they not only enthusiastically embrace future technologies, but also express their expectations about the looming avalanche of biotechnology drugs and their repercussions for reimbursement. Some of them even want to contribute to sustainable drugs discussions about access, adoption of drugs, cost-effectiveness, and maximum expenses per treatment per year against the backdrop of the finite resources of, for example, the tax-funded health care systems in the UK.²⁸

II. Debatable medical need (disease mongering): the case of lifestyle drugs

The second (unsustainable) drug innovation problem of debatable medical need concerns innovations that show profitability and feasibility but lack medical need, and includes the so-called ‘me-too’ products and therapies for diseases that are ‘constructed’ by society or companies. Moynihan and colleagues²⁹ call this ‘disease mongering’, in which actors envisage ordinary, mild, personal, or social ailments as medical problems. Flower³⁰ and Gilbert and colleagues¹¹ put these medicines on the agenda as ‘lifestyle drugs’. Examples include drugs for ADHD, irritable bowel syndrome, erectile dysfunction, shyness, and baldness,²⁹ but also obesity and metabolic syndrome.^{31,32,6}

A patient organization should contribute to the articulation and definition of the related medical condition in a scientific way in order to establish its identity and role. Sometimes the focus lies only on arguing why a condition should be regarded as a disease with related medical needs. For example, patients with erectile dysfunction (ED) have unique characteristics that are different from those of patients with other medical conditions, ED being heavily influenced by cultural and psychosocial factors. Barriers to seeking ED treatment (e.g. Viagra) were beliefs that ED was a normal part of ageing and that the condition would resolve on its own.^{33,34}

Another example are metabolic syndrome or obesity, which have clear symptoms that are quantitatively diagnosed. These conditions increase the risk for diabetes and cardiovascular diseases, for example. Although these correlations are beyond dispute,³⁵ some stakeholders in the health care arena do not regard metabolic syndrome or obesity itself as an illness,^{7,32,36} which is problematic for patient advocacy groups dealing with obesity. In case of an anti-obesity drug, regulatory and reimbursement agencies have emphasized prevention instead of pharmaceutical cures.

At the same time, and maybe in a perverse way, the indeterminate characteristics of a disease also enlarge the group of potential patients, making it easier for the related patient groups to appeal for more future drug R&D. In the case of ADHD, patient groups, health professionals, and science journalists proactively promoted the ADHD drug Ritalin, starting awareness campaigns and informing patients about this new drug via their websites.³⁷

⁶ It should be added that the degree to which these examples are in fact examples of disease mongering is the subject of discussion, which is at the same time precisely what we want to investigate here: the extent to which stakeholders try to influence these debates.

⁷ We explicitly want to refer to footnote 6 here.

Regarding the lifestyle disease Irritable Bowel Syndrome (IBS) and its treatment with Lotronex, patients organized themselves in the Lotronex Action Group to defend their interests.^{38,39} They lobbied for Lotronex to be put back on the market, after a late-2000 withdrawal, by bombarding both the drug producer GSK and the FDA with e-mails, letters, and calls about loss of quality of life through lack of the medicine. Patients were pleading at FDA hearings for re-approval of their medicines. Furthermore, the International Foundation for Functional Gastro-Intestinal Disorders lobbied for re-launch, showing patients and patient groups' involvement during the approval stage of lifestyle drug development. As the Foundation received funding from GSK³⁸ for expanding their research and teaching programmes on IBS, leading to more publicity about the disease IBS,⁴⁰ patient groups and sponsored advisory foundations were used for creating legitimacy for the disease and its treatment. At the same time, lawyers of patients with serious side-effects due to Lotronex were pleading against re-introduction on the market,⁴¹ trying to build up resistance against too positive expectations of Lotronex treatment.

Thus, some pharmaceutical companies make use of this indeterminate situation by creating patient groups by themselves or by funding organizations in order to influence disease advocacy.^{25,42} O'Donovan⁴³ called this "corporate colonisation". At the same time she warned against treating every patient organization as suspicious. Moreover, in some instances the lack of a clearly defined disease or the lifestyle or embarrassing character of a condition causes patient advocacy groups not to be formed. And when formed they might be deficient in voice, resources, or backing from medical experts.

This class of diseases is characterized by the fact that patients are blamed for their situation ('victim role') and that their condition is not necessarily recognized as a disease. There might be a mismatch between what patients find to be important medical needs and what society sees as significant. Therefore, patient organizations concentrate on defining disease, weighing medical argumentation and causalities underlying the disease definition. They also need to overcome organizational problems: attracting members and support from the medical profession. They do this by aligning with those scientists and pharmaceutical companies that do show an interest in these diseases, despite the debatable medical need. It can be concluded that patient organizations work on articulation and definition, but have difficulties in looking forward and linking to the drug innovation process. They are engaged in a rearguard fight by contributing to increasing understanding of a disease or ailment, broadening the discussions and overcoming resistance, creating a shared agenda about the importance of the disease, and discussing the mismatch between the disease and the cultural and societal norms.

III. Low economic profitability: the case of neuromuscular orphan drugs

The third sustainability issue of low economic profitability (in drug innovation) deals with disease areas that show medical need and feasibility but are economically uninteresting. Pharmaceutical companies are not eager to develop drugs for these diseases, and patients with these diseases are therefore called 'orphans'.⁴⁴ Orphan diseases include rare disorders which are life-threatening or chronically debilitating and have a low prevalence; and neglected disorders, which are prevalent in low-income countries.⁴⁵

Pharmaceutical companies and other involved stakeholders are not stimulated to invest in orphan drug R&D. Therefore, for these patients the need to voice their demands is paramount. This, together with the fact that small numbers of patients make it harder for patients to shy away from participating in patient representation, and the chronic and life-threatening character of the disease, leads to relatively well-organized and active patient advocacy groups that try to stimulate orphan drug R&D, translation of basic research into clinical practice, fund-raising, organizing international networks, and so on.⁴⁶

An example of an active patient group working on rare diseases is the Dutch Neuromuscular Diseases Association (VSN). This organization has as its objective the stimulation of research, amongst other means by propagating cooperation between academics and medical specialists on a national and international level and between the researchers and pharmaceutical companies. Moreover, the VSN is involved in the organization of clinical trials, managing a large patient database that pharmaceutical companies and researchers can only use if they comply with certain quality criteria. The VSN also tries to influence the development of particular drugs by contacting companies and researchers and bringing them together. Examples include (potential) drugs for Pompe disease and amyotrophic lateral sclerosis. Lastly, the patient group reckon the management of (high) expectations among their tasks. This management of expectations includes reacting to scientific news or proactively scanning scientific research, which leads to attempting to understand and validate the information in cooperation with scientists. Based on this, the organization articulates disclaimers and balancing of expectations with realistic views. They always leave the choice to the patients, and if patients, despite the warnings, want to adopt a new therapy, they encourage evaluation of safety and efficacy data. In this mechanism, visions about emerging technologies are created and maintained.

Patient advocacy is characterized by taking control of elements of drug R&D and the subsequent implementation on the market and informing as many actors as possible in order to engage them in problems around orphan drugs. Patients are poorly off and should bear the responsibility themselves. A crucial argument includes the right to be healthy, but this time even more so because patients with rare diseases are at the mercy of society, which might not have heard of the disease. Therefore, the emphasis lies on communication about rare diseases, strongly voicing demands that shift away from economic arguments to those stressing solidarity, and actively engaging in all stages of the innovation process as much as possible (a lot of prominent examples of patient participation, as illustrated in Section 3, come from the field of rare diseases). In this light, it is important to make use of new promises and expectations to create momentum in R&D (dynamics of expectations) and to underline the significance of orphan research for other, more common diseases. Moreover, the patient organizations attempt to contribute to discussions about ethical, legal, and social aspects of drug R&D by voicing their interests, but also by underlining solidarity and equity issues, in this way counteracting anti-technological positions such as those taken by animal rights groups.

5. Concluding remarks

The objective of this article is to show how patient advocacy organizations articulate and manage expectations about new pharmaceutical innovations in the context of problems

emblematic of unsustainable drug innovation. The stylized cases presented above have shown that different sustainability problems mean that patient organizations fulfil in their tasks differently with regard to advocacy and expectations. Table 2 summarizes this.

Table 2: Comparing dealing with expectations about different sustainability problems in drug innovation.

| Class | Focus of patient groups on innovation process (Figure 2) | Articulation of expectations | Management of expectations |
|--|--|--|--|
| I: Unequal access to medicines | Later stages (reimbursement) | Awareness of broader social context | Actively participate in context of societal debate |
| II: Debatable medical need (disease mongering) | – | No focus on future-oriented expectations; defensive | Alignment with other stakeholders; participate in societal debates |
| III: Low economic viability (orphanization) | All stages | Speed up and stimulate R&D (move to new frontiers); ethical, legal, and social aspects of drug R&D | Balancing enthusiasm with reality to avoid resistance and disappointment |

Table 2 illustrates that there are differences in the ways patient advocacy groups participate in the drug innovation process and articulate and manage expectations with regard to different sustainability problems with drug innovation. The most important ways of managing expectations are 1) achieving a balance between, on the one hand, making use of enthusiasm and the dynamics of expectations and, on the other hand, ‘forced forwarding’ or overemphasizing risks and 2) forming and communicating expectations and visions in the context of societal debates.

Scientists can either use or abuse patient advocacy groups. Abuse would mean that scientists prematurely present their findings as breakthroughs in order to mobilize patient groups to create more momentum and funds for their activities. On the other hand, if they interact together intensively and exchange useful knowledge, in this way producing balanced expectations about the future of sustainable pharmaceutical products and technologies, then these alliances are not easy to dismiss by other actors. These alliances can also determine well whether a technology is intrinsically novel and emergent (and needs financing or backing) or merely advertised as such as part of rhetoric efforts.²⁸

Policymakers might also benefit from patient groups that articulate and manage their expectations because this vision creation can have huge creative potential and at least communicates the patients’ experiential knowledge and eagerness to champion certain new technologies in societal debates about a sustainable drug system. At the same time, these policymakers should be aware of and able to deal with the heterogeneity of the patient organizations. This heterogeneity is often seen as a weakness (patients cannot produce one vision) but can also be regarded as a strength, enriching and broadening the societal debate.

Concerning patient groups, three critical points should be taken into account. Firstly, patient groups are not the same as individual patients, so there might be a problem with the validity of representation. Secondly, patient groups are constantly positioning themselves towards the new technology and towards other actors. This is a fine balancing act. For example, patient groups can benefit from pharmaceutical company

support in order to stress medical need, but at the same time should remain critical towards this company – or at least maintain a certain level of objectivity – because otherwise other parties would not regard the patient group as a serious and full player in the field. Thirdly, patient advocacy groups form a heterogeneous class of organizations. They show large variety in the way they organize their advocacy activities across the three sets of sustainability problems of drug innovation. Moreover, within these sets and in different phases in time, these organizations demonstrate different behaviours. This variability makes dealing with patient groups complex and the lack of clarity and antinomy that is inherent to this mosaic of advocacy might position patient groups as incomprehensible. On the other hand, the heterogeneity leads to broadening and enriching the debates on sustainability of drug innovation and to differences in attention to certain issues, for example, ethical and social impacts, access, and solidarity, which make these organizations underestimated vehicles for the articulation of expectations.

Therefore, it remains important to leave the debate around drug research, development, and usage accessible to all relevant parties, among which patient advocacy groups are valuable ones. Moreover, it is also important to set up this assessment of drug development in early stages of development because then the characteristics of this technology are still malleable.

By focusing on the roles and expectations of patient advocacy, this paper gives a first insight into how to develop an alternate, ideal pharmaceutical system that provides a solution to unequal access, debatable medical need, and low economic profitability (unmet medical need), thereby helping the health care system to maintain a sufficient level of sustainability.

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