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Unmet Medical Need as a Driver for Pharmaceutical Sciences − A Survey Among Scientists

Z. Kusynová[a,b], G.M. Pauletti[b,c], H.A van den Ham[a,*], H.G.M. Leufkens[a], A.K. Mantel-Teeuwisse[a]

[a] Utrecht Centre for Pharmaceutical Policy and Regulation, Division of Pharmacoepidemiology and Clinical Pharmacology, Utrecht Institute for Pharmaceutical Sciences (UIPS), Utrecht University, Utrecht, the Netherlands
[b] International Pharmaceutical Federation (FIP), The Hague, the Netherlands
[c] St. Louis College of Pharmacy, St. Louis, Missouri, United States

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ABSTRACT

Historical antecedents of pharmaceutical sciences are sound on product orientation based on (analytical) chemistry, drug delivery and basic pharmacology. Over the last decades we have seen a transition towards a stronger disease orientation. This raises questions on whether, how and to what extent unmet medical need (UMN) is important in priority setting, funding and impact in pharmaceutical sciences. An online survey in 2020 collected perspectives of internationally recognised pharmaceutical scientists ($N = 92$), mainly from academia and industry, on drivers and influencing factors in pharmaceutical sciences. The study offers a unique global perspective, demonstrating a solid command of the global needs in pharmaceutical sciences. The survey revealed that UMN is currently seen as one of the three most important drivers, also in addition to emerging trends in science and opportunities driven by collaboration. There are expectations that UMN's impact becomes more influential. This was consistent for both industry and academic respondents. The majority of respondents also indicated that anticipated lessons learned from COVID-19 will strengthen the impact of UMN on science and leadership. This is important as prioritisation of research towards UMN can address the clinical needs where needed the most.

Introduction

Historically, pharmaceutical innovation tended to be focused on the pharmacological level, where results of scientists' preferred research areas were, eventually, leading to approved treatment options. Major breakthroughs in pharmaceutical sciences in the past century were primarily driven by advances in medicinal and analytical chemistry, pharmaceutics, cell biology or receptor pharmacology.[1] Prior to the early 1960s, there were no safe and effective therapies for common illnesses such as atherosclerosis and essential hypertension, that carry an increased risk for of premature death.[2] Similarly, no such treatments were available for a whole host of incapacitating and fatal infectious and parasitic diseases that have affected many millions, especially in low- and middle- income countries.[3] Indeed, the majority of today's most generally prescribed medications such as the calcium channel blocking agents, statins, oral contraceptives, and bisphosphonates were after the 60s introduced as new therapies, alongside with advancements in many other therapeutical classes, such as antibiotics and antimalarials.[4] Surgical treatment of peptic and duodenal ulcers despite its associated risks was relatively common before the introduction of the proton pump inhibitors.[5]

Many more examples of how in the past, pharmaceutical scientists have been fundamental in enabling innovative therapeutic options for patients regionally and globally.[6] But many of these advances started rather at the lab bench than in the clinic. In the 1990s 'Drugs looking for a disease' was still a well-established concept.[7] Danhof et al. coined a paradigm shift from chemistry-biology based to pathology-of-disease medicine occurring around 2010.[8] With further continuous discoveries in disease-causing mechanisms, translating the findings into patients' unmet medical needs remained a challenge, but became a desired goal.[9,10]

Unmet medical need (UMN) is a widely used term in the healthcare sector with no single universal definition. Definitions of UNM in literature include (impact of) available treatments, patient population size or disease severity.[11] For the purpose of this study we defined UMN simply as medical needs in society (societal values) to be

* Corresponding author at: Utrecht University, Utrecht Centre for Pharmaceutical Policy and Regulation, Division Pharmacoepidemiology & Clinical Pharmacology, PO Box 800 82, 3508 TB, Utrecht, the Netherlands.
E-mail address: h.a.vandenham@uu.nl (H.A. van den Ham).

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addressed by scientists by providing a therapy where none exists (e.g. pandemics, orphan diseases) or providing a therapy which may be potentially better than available alternatives (e.g. antimicrobial resistance).[12] While addressing UMN has always been a topic of some importance for pharmaceutical scientists since the beginning of their existence, it was mainly implicitly and not as a primary driver.[1,10,13,14] But that picture is changing − and questions on whether, how and to what extent UMN is important in priority setting, funding and impact in pharmaceutical sciences are raised.

In order to answer these, we asked pharmaceutical scientists for their perspectives. Pharmaceutical scientists as a group cover a heterogenous mixture of expertise and training in many aspects of science and technology related to medical products.[15] This heterogenicity includes [but is not limited to] discovery, development, manufacturing, regulation, and utilisation of medical products - embracing how medicines work, how safe and effective products are brought to the market, their impact on the body and their effect on the prevention and treatment of disease.[15] Pharmaceutical scientists are mainly employed by academia and the pharmaceutical industry, but increasingly also by regulatory authorities, non-governmental organisations (NGOs) or public-private entities.[16,17] While (pre-clinical phase) basic discovery research is significantly led by academia and public institutions and funded primarily by government or by philanthropic organisations, late-stage (clinical phase) development is mainly led and funded by pharmaceutical industry.[18] Pharmaceutical industry is also typically more involved in manufacturing and quality assurance.[17,18] And industry is much more driven by business opportunities and targets compared to academia.[17]

Steering the focus of pharmaceutical sciences towards UMN is essential to address the clinical needs where needed the most. Addressing the above-mentioned questions on the how and what of UMN, this study offers a unique global insight in perspectives of experienced pharmaceutical scientists from various countries and backgrounds on the current environment of pharmaceutical sciences, main factors affecting priority setting and how these may change in the future, and on whether and how UMN has a role in all this. In addition, the study was conducted amid the acute global UMN of COVID-19 disease. This gave us the opportunity to mirror our findings in the context of this dramatic global event.

Methods

Setting and Participants

In the summer of 2020, an online survey was sent to speakers and/or chairs ($n = 380$) who participated in the past three Pharmaceutical Sciences World Congresses (PSWC), organised by the International Pharmaceutical Federation (FIP), in Montréal (2020, virtual), Stockholm (2017), and Melbourne (2014). FIP is the global umbrella federation representing pharmacists and pharmaceutical scientists worldwide. We selected this group for our study, as PSWC speakers and/or chairs are selected by the International Scientific Programming Committee based on their international merit and recognised leadership in pharmaceutical sciences worldwide. We expected to collect a well thought-out set of views and perspectives from this group.

Data Collection

The survey was created using LimeSurvey® software and consisted of 14 multiple-choice questions and several open questions, divided into 3 sections. The first section of 5 questions covered general information about the participants (e.g., name, gender, age, country) and their background (pharmaceutical sciences specialty area, position, education background, years of experience). Furthermore, participants were asked to indicate the current closest match of their affiliation(s).

The second section of the survey concerned the perspectives on pharmaceutical sciences and consisted of questions on a 5-point Likert scale covering: most influential factors on research focus, most important drivers of pharmaceutical sciences research and most important factors shaping the future of pharmaceutical sciences. UMN was included in each of the questions as one of the options out of 8-9 pre-defined options and all factors were presented in an equal, random, manner. Given there is no one definition of UMN, different wording was used to indicate (unmet) medical need: unmet medical needs; medical need, societal values; and medical needs, pandemics and these responses were categorised as being related to UMN. The full wording of the survey questions is included in the Supplementary file 1.

Given the pertinent and global UMN of COVID-19 disease, we also asked an open question about how the COVID-19 pandemic may change pharmaceutical sciences now and in the future.

Data Analysis

We compared the demographic (gender, age, country) and professional (education background, affiliation, pharmaceutical sciences specialty area, position) profile of the respondents with the demographic profile of the speakers and chairs of PSWC we reached out to.

Questions with 5-scale Likert scale answer options were analysed quantitatively using Microsoft Excel 2016 and descriptive statistics were calculated. Responses were converted into a numerical scale with 1 point allocated to strongly disagree/not at all (or equivalent wording) and 5 points to strongly agree/very likely (or equivalent wording) and were analysed describing frequencies. Next, the mean and standard deviation were calculated to examine whether the items contributed equally to the total scale score. Furthermore, we compared responses from the most prevalent groups in the survey, i.e. participants coming from academia and from pharmaceutical industry, using descriptive statistics and analysing describing frequencies, as well as calculating the mean and standard deviation to examine whether the items contributed equally to the total scale score. The answers on open questions were organised into prevalent themes and analysed accordingly.

Results

Demography

We received, after two reminders, 92 responses out of 380 surveys sent (24% response rate). The demographics (gender, country) and professional (education background, pharmaceutical sciences specialty area, position, years of experience) profile of the respondents are displayed in Table 1.

The demographic profile of the respondents corresponded with the demographic profile of the original group we reached out to (Table 1). Most of the participants' affiliations were from academia ($n = 64$) and the industry ($n = 17$). These were the two most represented groups. Among those, a small fraction (<5%) of respondents indicated affiliation to both academia and industry. A small number of participants indicated the following sectors − solely or in combination with another affiliation: non-governmental institution ($n = 6$); governmental institution, international body and healthcare (each $n = 5$). There were four participants from private research institutions, three participants from a philanthropic foundation, or charity and one from regulatory, quality control. Similarly, the original group of all invited respondents consisted mostly of academia ($n = 242$, 64%) and the industry ($n = 54$, 14%) as the biggest represented groups.

Table 1
Descriptive Statistics for the Pharmaceutical Scientists who Participated in the Study ($n = 92$) and in the Originally Surveyed Group ($n = 380$).

	Respondents No. (%)	Surveyed group No. (%)
Gender		
Female	25 (27)	96 (25)
Male	64 (70)	284 (75)
Not disclosed	3 (3)	0 (0)
Geographical region[a]		
European Region	37 (40)	145 (38)
Region of the Americas	30 (33)	136 (36)
Western Pacific Region	19 (21)	88 (23)
Other	6 (6)	11 (3)
Academic rank / professional position		
Full professor	42 (46)	
Research director, management lead	19 (21)	
Associate professor, senior researcher	12 (13)	
Postdoc, junior researcher	8 (9)	
PhD student	6 (7)	
Other	5 (5)	
Educational background (highest degree)		
Pharmaceutical sciences	43 (47)	
Pharmacy	22 (24)	
(Medicinal) chemistry	5 (5)	
Biology, biotechnology	5 (5)	
Medicine, epidemiology	3 (3)	
Biophysics/physics	3 (3)	
Other (e.g., data science, humanities, etc.)	11 (12)	
Pharmaceutical sciences specialty area (currently active in)		
Drug formulation, pharmaceutics	29 (32)	
Clinical pharmacology, drug development	17 (18)	
Health systems, policy, regulation	13 (14)	
Clinical pharmacy, pharmacy practice	10 (11)	
(Cell) biology, systems biology, disease models	8 (9)	
(Medicinal) chemistry, drug discovery	6 (7)	
Pharmacology, drug action	5 (5)	
Analytical sciences and quality control	4 (4)	
Years of experience		
40+	20 (22)	
30+	22 (24)	
20+	17 (18)	
10+	12 (13)	
≤10	21 (23)	

[a] Based on World Health Organisation (WHO) regions.

Perspectives on the Drivers in Pharmaceutical Sciences

We asked the survey respondents about ‘How important are the following aspects in your research?’ In Fig. 1 the results of this question are summarised indicating that multidisciplinary collaboration, access to technology (and data) and unmet medical need (UMN) and societal values are in the top three of most important drivers influencing how the pharmaceutical sciences are shaped. Interestingly, factors like funding opportunities, patents or interactions with students were seen as less relevant.

In Table 2 the same question is addressed, showing the three most relevant drivers for all respondents, stratified for academia and industry.

For respondents from academia, UMN was the fourth most influential, surpassed by multidisciplinary collaboration and access to technology (and data) but also publications, and citations. Industry respondents indicated UMN at third place, surpassed by the same two factors of multidisciplinary collaboration and access to technology (and data). We did the same for ranking the top three most influential factors when choosing research focus, also stratified for academia and industry (Table 3). Also here intrinsic scientific factors (i.e. trends in science, UMN and collaboration) surpassed other factors.

We also asked the respondents about their views on the future of pharmaceutical sciences, with overall, and stratified for academia and industry, strong agreement on UMN as being the most critical factor to shape pharmaceutical sciences (Table 4). Also available technologies and breakthrough successes were seen as convincing building blocks for the future.

When asked to identify three contemporary research questions or areas in the pharmaceutical sciences where one would invest in if one would get a million EUR/USD grant, we received a total of 224 ideas - 93% of those were directly or indirectly related to solving UMN. This included suggestions directly related to solve UMN using translational science applications (e.g., drug delivery, gene & cell therapy, personalised medicines, nanomedicines, etc., $n = 75$, 36%) and various clinical areas (e.g., rare diseases, communicable diseases, non-communicable diseases, etc., $n = 66$, 32%). Furthermore, 68 (38%) suggestions were related to UMN indirectly, through improvement of available therapies or improvement of access to medicines, themed mostly around: technology (e.g. use of technology and computing power for drug discovery, etc.; $n = 17$, 8%), but also in areas of policy/regulation (e.g., earlier access to innovative drug products, improved clinical trial design, etc., $n = 21$, 10%), practice/care systems (e.g., improved patient outcomes, minimising adverse drug effects, $n = 19$, 9%), and basic sciences (epidemiology, immunology, pathway biology, mathematical modelling, $n = 11$, 5%). Fifteen answers (7%) were not at all related to UMN, with topics around education, increasing the efficiency of health services, environmental impact of medicines, for example.

Perspectives on COVID-19 and its Effect on Other Unmet Medical Needs

In response to the open question of how eminent scientists thought the COVID-19 pandemic will change pharmaceutical

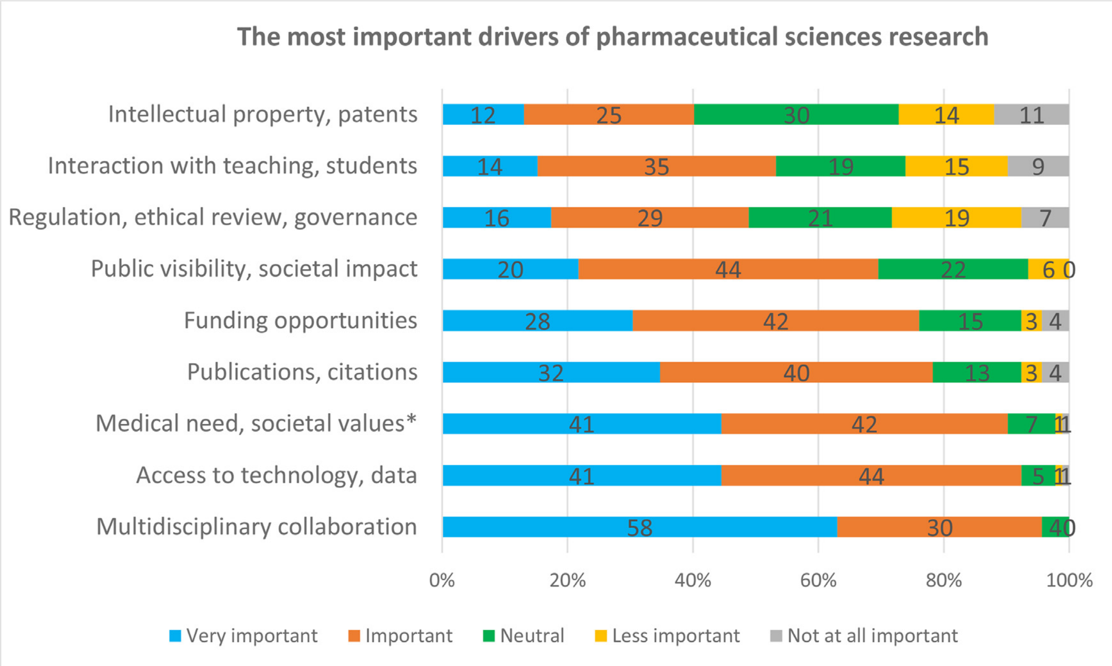

Fig. 1. The most important drivers of pharmaceutical sciences research.
*Response categorised under unmet medical need (UMN).

Table 2
The Most Important Drivers of Pharmaceutical Sciences Research.

Drivers:	Nr. order [a]	Mean (SD) [b]	Very important, No. (%)	Important, No. (%)	Neutral, No. (%)	Less important, No. (%)	Not at all important, No. (%)
Multidisciplinary collaboration[c]	#1	4.6 (0.6)	58 (63)	30 (33)	4 (4)	0 (0)	0 (0)
Academia	#1	4.6 (0.5)	41 (64)	21 (33)	2 (3)	0 (0)	0 (0)
Industry	#1	4.5 (0.6)	10 (59)	6 (35)	1 (6)	0 (0)	0 (0)
Access to technology, data[c]	#2	4.3 (0.8)	41 (45)	44 (48)	5 (5)	1 (1)	1 (1)
Academia	#2	4.4 (0.7)	29 (45)	32 (50)	2 (3)	0 (0)	1 (2)
Industry	#2	4.4 (0.6)	8 (47)	8 (47)	1 (6)	0 (0)	0 (0)
Unmet medical need[d]	#3	4.3 (0.9)	41 (45)	42 (46)	7 (8)	1 (1)	1 (1)
Academia	#4	4.3 (0.7)	26 (41)	34 (53)	3 (5)	0 (0)	1 (2)
Industry	#3	4.2 (0.9)	8 (47)	6 (35)	2 (12)	1 (6)	0 (0)

[a] Order of the drivers that scored highest in the category very important, out of 9 drivers.
[b] Based on a 5-point Likert Scale on which 1 = not at all important and 5=very important.
[c] 92 participants answered.
[d] Survey wording used was “Medical need, societal values”
Abbreviations: SD = standard deviation.

Table 3
The Most Influential Factors When Choosing a Research Focus in Pharmaceutical Sciences.

Research focus choice factors:	Nr. order [a]	Mean (SD) [b]	Very influential, No. (%)	Influential, No. (%)	Neutral, No. (%)	Rather not influential, No. (%)	Not at all influential, No. (%)
Emerging trends in science[c]	#2	4.0 (0.9)	27 (29)	46 (50)	12 (13)	5 (5)	2 (2)
Academia	#2	4.0 (0.8)	17 (27)	36 (56)	8 (13)	3 (5)	0 (0)
Industry	#3	3.6 (1.3)	5 (29)	7 (41)	1 (6)	2 (12)	2 (12)
Unmet medical need[c, d]	#1	3.9 (1.1)	31 (34)	35 (38)	14 (15)	7 (8)	5 (5)
Academia	#1	3.9 (1.1)	22 (34)	24 (38)	11 (17)	5 (8)	2 (3)
Industry	#2	3.8 (1.3)	6 (35)	7 (41)	1 (6)	1 (6)	2 (12)
Opportunities driven by collaboration[c]	#3	3.8 (0.9)	20 (22)	49 (53)	15 (16)	5 (5)	3 (3)
Academia	#3	3.9 (0.9)	14 (22)	39 (61)	5 (8)	4 (6)	2 (3)
Industry	#4	3.9 (0.8)	4 (24)	7 (41)	6 (35)	0 (0)	0 (0)

[a] Order of the factors that scored highest in the very influential category, out of 8 factors.
[b] Based on a 5-point Likert Scale on which 1 = not at influential and 5=very influential.
[c] 92 participants answered.
[d] Survey wording used was “Unmet medical needs (e.g., COVID-19, orphan diseases, antimicrobial resistance)”.
Abbreviations: SD = standard deviation.

Table 4
The Most Important Factors Shaping Future of Pharmaceutical Sciences.

Factors shaping future of pharmaceutical sciences:	Nr. order [a]	Mean (SD) [b]	Strongly agree, No. (%)	Agree, No. (%)	Neutral, No. (%)	Disagree, No. (%)	Strongly disagree, No. (%)
Unmet medical need[d]	#1	4.4 (0.8)	48 (52)	38 (41)	3 (3)	2 (2)	1 (1)
Academia	#2	4.4 (0.6)	29 (45)	32 (50)	2 (3)	1 (2)	0 (0)
Industry	#1	4.5 (0.7)	11 (65)	5 (29)	1 6()	0 (0)	0 (0)
Technology in preventing and treating disease [c]	#2	4.3 (0.8)	46 (50)	35 (38)	8 (9)	1 (1)	2 (2)
Academia	#1	4.3 (1.0)	31 (48)	24 (38)	6 (9)	1 (2)	2 (3)
Industry	#2	4.4 (0.7)	10 (59)	6 (35)	1 (6)	0 (0)	0 (0)
Breakthroughs / successes[c]	#3	4.2 (0.9)	38 (41)	42 (46)	7 (8)	3 (3)	2 (2)
Academia	#3	4.1 (1.0)	26 (41)	27 (42)	7 (11)	2 (3)	2 (3)
Industry	#3	4.5 (0.5)	9 (53)	8 (47)	0 (0)	0 (0)	0 (0)

[a] Order of the frequency of the of the factors that scored the highest in the strongly agree, out of 8.
[b] Based on a 5-point Likert Scale on which 1 = strongly disagree and 5=strongly agree.
[c] 92 participants answered.
[d] Survey wording used was “Medical needs, pandemics”.
Abbreviations: SD = standard deviation.

sciences now and in the future in their region or globally, there were 60 respondents (65%) that indicated the COVID-19 pandemic will change pharmaceutical sciences. The COVID-19 pandemic was perceived as impactful in the near future. There were 10% ($n = 9$) respondents who indicated no visible impact at this stage and 25% ($n = 23$) did not answer this question.

The respondents gave their perspectives on the lessons learned on UMN for pharmaceutical sciences from the COVID-19 disease. Five themes, i.e. Reprioritisation of funding, Drug development: new research tools & study designs, Regulatory procedures optimisation, Interdisciplinary teamwork and Public engagement and advocacy, emerged when analysing the perspectives given etc. Table 5 summarises these perspectives and provides multiple examples for each theme.

Discussion

Prioritising the focus of pharmaceutical sciences towards UMN is critical to address the patients' needs where needed the most. The study results demonstrate a solid command of the global UMN in pharmaceutical sciences, confirmed through a unique perspective of

Table 5
Impact of COVID-19 on Pharmaceutical Sciences Through the Lens of Tackling Future Unmet Medical Need (UMN).

Area	Projections
1 Reprioritisation of funding	Paradigm shift: fast re-prioritisation and mobilisation of funding towards UMN at global level is possible. Infectious diseases research to strengthen (learning from recent epidemics, i.e., severe acute respiratory syndrome (SARS), Ebola, Zika, COVID-19). Resources to redirect towards epidemic preparedness and fighting threats, such as antimicrobial resistance (i.e., finding new antibiotics). UMN as a driver of scientific leadership to gain importance, long-term including UNM outside of infectious diseases.
2 Drug development: new research tools & study designs	Drug development to become speedier and more efficient with new research tools, innovative study designs, interdisciplinary teamwork, optimised regulatory procedures. Basic sciences, such as chemistry for small molecules, to gain prominence in drug discovery. Social sciences to increase presence in pharmaceutical sciences. Landscape of clinical trial phases I-III typically pursued in the drug development chain − to conditional on priority UMN and applied to UNM affecting small populations (e.g., rare diseases). Innovation progress in one UMN applied to stimulate progress in other UMN (accelerating pharmaceutical research in ribonucleic acid (RNA) therapies for COVID-19 vaccines, and vice versa learning from COVID-19 for other areas such as cancer therapies and brining more advanced therapies to more patients). Mechanisms in place to prevent severe restrictions/interruptions in the supply chains across countries: pharmaceutical supply chain research to increase and speed up, e.g., distributed manufacturing (continuous flow modular manufacturing, etc.) accepted and pursued more broadly, as well as increased local production.
3 Regulatory procedures optimisation	Pace of regulatory approval to increase. Less stringent regulation criteria for clinical trials and reduced animal trials. Once adopted in post-COVID-19 regulatory frameworks, the drug development and good practices in procurement and supply to be re-evaluated and strengthened to meet the UMN and overcome challenges faster. Regulation criteria and support to embrace scientific progress and gain of knowledge in the context of uncertainty (i.e., relative truth vs. absolute truth in scientific investigation, importance of lessons learnt for overall scientific progress).
4 Interdisciplinary teamwork	Greater willingness to cooperate, both by sharing expertise and sharing materials and equipment. More multidisciplinary and multi-stakeholder collaboration to further ensure the academic research environment are applied to other UMN. Information technologies and communication strategies disciplines to better integrate in pharmaceutical sciences.
5 Public engagement and advocacy	Scientific leadership driven by UMN to align prioritisation among all stakeholders. All stakeholders (public, private industry, policy makers, payers, etc.) to become more interested in solving UMN and related social healthcare problems, strengthening of health systems, preparedness for outbreaks, global responsibility, and urgent need for international collaboration in tackling UMN. Scientists to better communicate the scientific advances addressing UMN to the public. Public to become critical in engagement in the scientific discourse around UMN (e.g., adoption of Schrödinger's uncertainty principle). More emphasis to be given to patient engagement and self-care on prevention side, as well as personalised health care on treatment side.

pharmaceutical scientists from around the world. In addition to being the current influential driver, there are expectations on further increase of UMN influence in driving pharmaceutical sciences. This was also confirmed by looking specifically at the case of UMN in the context of the COVID-19 pandemic, where the majority of respondents indicated COVID-19 being a catalyst of strengthening the influence of UNM in shaping the future of pharmaceutical sciences. They anticipated lessons learned from COVID-19 will strengthen the impact of UMN on science, which will in turn enhance sciences' leadership to help address the UMN. Interestingly, responses by the academia and industry were aligned on prioritising UMN. A small difference to highlight was that industry participants indicated UMN most frequently as the second most influential factor, surpassed by the research policy/strategy of the institute or company.

UMN was also dominating the research choice in the open question as well, where nearly all of respondents' suggestions for contemporary research questions or areas in the pharmaceutical sciences (if given a generous, one million EUR/USD grant for any research of their choice) were related to solving UMN − by typically closing a research gap in various clinical / diseases areas, improvement of existing therapies (dosage, absorption, safety, quality, personalised therapy, etc.), innovation (use of new technologies, improving pharmaceutical services) and/or modernisation of the research / regulatory environment.

In the past, pharmaceutical sciences in their focus on basic sciences and product orientation had an ambiguous relationship to UMN. However, that picture is changing with UMN becoming the main driver of the direction and focus of pharmaceutical sciences[1,8]. Also the results of this study support that transition. Still we see mixed perspectives in the literature. On one hand, available data on research funding and investment are supporting the notion that UMN is driving the pharmaceutical sciences.[19,20,21,22] On the other hand, there are several studies that have pointed out that pharmaceutical sciences are reactive rather than proactive to UMN, where focus and funding of clinical research particularly by the pharmaceutical industry is strongly associated with commercial viability, being attentive to UMN particularly through market analysis.[23,24] This may not be a sustainable way forward to tackle UMN, due to skyrocketing costs of R&D and plummeting innovation productivity (caused largely by increased complexities of therapy targets and stringent regulatory demands).[25] Lack of proper or systematic funding is particularly apparent in the niche areas of UMN, i.e. rare or neglected tropical diseases, where return of investment is low and many priority research gaps still exist.[26] Therefore, it comes as a surprise that the availability of funding was not considered to be of particular importance for respondents with an academic nor with an industry background.

Nonetheless, UMN can be found in a plethora of non-orphan and non-neglected disease categories, where funding is not the main reason for stagnation, but it is the scientific bottlenecks who are the main contributors to underperformance. Alzheimer's disease, dementia, cardiovascular disease, chronic pain conditions, osteoporosis, are but a few examples of areas with little progress despite relatively generous funding.[27,28] Importantly, it is not only neglected diseases where UMN remains, but many diseases of high-income countries are also affected.[9] Globalisation of diseases (communicable diseases like COVID-19, AIDS, SARS etc.) as well as wide spread of non-communicable diseases for which there is still no cure (cancer, dementia, etc.) is exacerbating the problem overall, in high-, middle- and low-income countries alike. UMN remains both in large indications (e.g., Alzheimer's disease) and in niche indications (e.g., Huntington disease).[29]

This is where COVID-19 can show example of unprecedented coordinated and speedy efforts − both in mobilising available scientific information (e.g., learning from m-RNA therapies initially used for cancer) and implementing supporting measures (e.g., removing obstacles to the free flow of research data and ideas).[30,31,32] In line with the opinions expressed by our respondents, lessons can be learned from finding and distributing COVID-19 therapeutics and vaccine and from evidence-informed planning to overcoming accompanying challenges. The hope is that these lessons, for example, the benefits of scientists-led multi-stakeholder collaboration, extensive communication of scientific advances, removing regulatory barriers, etc. will eventually bring more advanced therapies to more patients. However, it may be necessary to wait until the pandemic is over to allow objectively examining the effect of COVID-19 pandemic. Also, more research is needed to analyse the full spectrum applicable to other UMN. Table 5 which outlined five thematic areas can serve as the starting point for the development of such analysis.

As UMN gains importance in pharmaceutical sciences and research priority setting, some standardisation is needed for its scope. UMN can mean different things to different people and also for the purpose of this study, different wording was used to indicate UMN. Lu et al pointed out that while "UMN" has important research prioritisation and regulatory implications, there is no empirical analysis of its real world usage and it currently describes both rare indications with no or few treatment options, as well as clinically indolent, commonly occurring indications.[33] Sandman and Hofmann (2019) criticised UMN being used to feed into criteria guiding research priority decision-making without underlying structured conceptual and normative considerations.[34] Further deliberations on the role of UMN as a driver remains important, especially given recent reflections on drivers and enablers of adaptive drug development pathways, where, as argued by Eichler et al (2015), one should not separate UMN from other value elements regardless of the existing need. Therefore, UMN alone should not drive decisions on prioritisation.[35]

Albeit we could observe a clear priority for UMN among our survey respondents, we should not forget that basic, curiosity driven pharmaceutical research without a clear view on expected benefits for society and health systems, also may deliver many years later. Our findings should not be interpreted as a plea for a defund of basic research. We also acknowledge that opinions of our survey respondents, who are chairing or speaking at a global conference, may not reflect the opinions of many other pharmaceutical scientists, especially young researchers. Albeit that the survey was conducted in a population of a primarily established generation of pharmaceutical researchers, we could find a shift towards more clinical and UMN research topics. This finding indicates that the future will show more to come on UMN when younger generations will fly in. Further studies are needed to explore the opinions in different groups that are outside of the scope of this study.

Overall, this study highlights that pharmaceutical scientists consider UMN being one of the major driving forces of pharmaceutical sciences, also in addition to emerging trends in science and opportunities driven by collaboration. They anticipate that the role of UMN will strengthen in the future. As a matter of a fact, through demonstrating UMN being the driving force in pharmaceutical sciences, pharmaceutical scientists can take leadership to better articulate the benefits for all stakeholders (for patients / payers / industry), show how real needs are being met by science, and advocate for orientating the funds given to research towards real needs. The findings in this study can support these efforts, for example they can be employed in the development of vision and strategic planning for addressing UMN as main driver for pharmaceutical sciences and research, both in academia and in industry. Some question whether the direction of pharmaceutical sciences is aligned with what society needs today. Therefore, demonstrating UMN being the driving force in pharmaceutical sciences can help pharmaceutical scientists take the leadership in this timely endeavour to bring more advanced therapies to more patients.

Strengths and limitations

This study gathered perspectives from pharmaceutical scientists, with international merit, and recognised leadership in pharmaceutical sciences worldwide, contributing to the strengths of this study. There are several limitations to note. First, there was a relatively small sample size identified by a single international pharmaceutical federation and some geographical regions were underrepresented, as well as the opinions of general researchers, and especially young researchers might not be reflected. Secondly, this was limited to experts from the pharmaceutical sciences largely coming from academia and industry. Thirdly, the survey was running during the COVID-19 pandemic which may have negatively impacted the response rate. And finally, different wording related to (unmet) medical needs of the society was used to describe UMN, given there is no universal definition of UMN. While these limitations can constrain generalisability, the methods provided a rich depth of information and promoted trustworthiness of findings. Clear and consistent themes emerged, representing expert viewpoints from various regions and diverse specialisations within the pharmaceutical sciences.

Conclusions

According to perspectives of pharmaceutical scientists involved in this study, with backgrounds from both academia and industry, UMN is increasingly a driver for the direction of pharmaceutical sciences. Majority of participants anticipate COVID-19 disease, being an UMN itself, will strengthen the driving force of UMN in pharmaceutical sciences in the future to address the clinical needs where needed the most.

Declarations of Interest

None. The views expressed in this article are the personal views of the authors and must not be understood or quoted as being made on behalf of the International Pharmaceutical Federation.

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Supplementary materials

Supplementary material associated with this article can be found in the online version at doi:10.1016/j.xphs.2021.10.002.

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