European Federation of Allergy and Airways Diseases Patients' Associations

# EFA's response to the public consultation on the EU Pharmaceutical Strategy (Directorate General for Health and Food Safety)

The European Federation of Allergy and Airways Diseases Patients' Associations (EFA) is the voice of over 200 million people living with allergy, asthma, and chronic obstructive pulmonary disease (COPD) in Europe. We bring together 39 national associations from 24 countries and channel their knowledge and demands to the European institutions. We connect European stakeholders to ignite change and bridge the policy gaps on allergy and airways diseases so that patients live uncompromised lives, have the right and access to the best quality care and a safe environment.

EFA is full Member of the European Patients' Forum and has contributed to the EPF's submission to this consultation, which we fully support.

EFA welcomes the opportunity to comment on the upcoming EU Pharmaceutical Strategy, considering it the necessary and welcome development, given the EU role and competences in the area of health. This document has been drafted as an attachment to the Online Questionnaire of the related consultation.

## Contents



## Access to affordable medicines

### Shortages of medicines and medical equipment in the EU

Equal access to medicinal products and equipment in Europe is a vast issue affecting patients. Shortages depend on a complex network of factors such as the level of demand, the market authorisation process, manufacturing/distribution issues, competition, national regulations on product quality, company decisions, the availability of equivalents, the economic situation, but also

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extraordinary political events[1]. Naturally, shortages of medicines translate into limited treatment availability and patients missing the care they need.

In the EU and worldwide, shortages of medicines represent a growing problem, resulting in ever widening unmet needs, limited patient access to necessary treatments (with the health risks that this entails), as well as disruptions in the functioning of national health systems[2,3]. This reality has been acknowledged by all major EU institutions[4,5] and the European Medicines Agency[6].

As a European community, EFA has serious concerns when a medicine is not authorized following the centralised process of the European Medicines Agency. The main problem lies in that when shortages occur, no matter the reason, there is no way to have a full overview of the situation across the region. This essentially relays the problem to the patients at the national level.

Furthermore, the **availability of a medicine does not ensure its accessibility**. Firstly, there is pricing setting at national level, in many cases adopted in a non-transparent and random way and with very little patient involvement. Secondly, reimbursement policies vary across and intra country, depending on payers and insurers. Thirdly, even when a medicine is reimbursed at a high percentage, patients might not be in a position to financially afford them.

Shortages can also result from **medicine withdrawals from the market** due to multiple causes. For example, it could be due to unintended health effects from the use of medicine despite it having been tested before; the revision of quality requirements by a public authority; inefficient commercial profit, or also as a result of a more efficient drug becoming available. Withdrawals do not always respond to patient needs and outcomes.

There is a great variety of actions that could be taken by the EU to prevent and/or anticipate shortages, while being consistent with its competences to protect public health and regulate the functioning of the Single Market. For example, disruptions in the supply of medicines could be mitigated by establishing communication channels for the industry to systematically inform EU and national authorities of potential problems in the supply chain. Such an alert system would help EU and national authorities prepare against shortages in advance. Furthermore, the acceleration in the implementation of ePrescriptions (in line with the provisions of the Cross-border Healthcare Directive[7]) would, amongst others, benefit healthcare systems plan their medicines stocks in due time.

In EFA's view, the new EU pharmaceutical strategy could help reduce shortages of medicines and medical equipment through a number of actions, including:

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[1] U.M. Musazzi, D. Di Giorgio, P. Minghetti ''New regulatory strategies to manage medicines shortages in Europe'', April 2020, https://www.ncbi.nlm.nih.gov/pmc/articles/PMC7125892/

[2] JAMA Network, ''European Medicines Agency Tackles Potential Shortages of Drugs for Treating COVID-19'', April 2020, https://jamanetwork.com/channels/health-forum/fullarticle/2765059

[3] U.M. Musazzi, D. Di Giorgio, P. Minghetti ''New regulatory strategies to manage medicines shortages in Europe'', April 2020, https://www.ncbi.nlm.nih.gov/pmc/articles/PMC7125892/

[4] European Parliament Briefing, ''Addressing shortages of medicines'', April 2020, https://www.europarl.europa.eu/RegData/etudes/BRIE/2020/649402/EPRS_BRI(2020)649402_EN.pdf

[5] Council conclusions on strengthening the balance in the pharmaceutical systems in the European Union and its Member States, 2016 https://eur-lex.europa.eu/legal-content/EN/TXT/?qid=1581347403221&uri=CELEX:52016XG0723(03)

[6] European Medicines Agency https://www.ema.europa.eu/en/human-regulatory/post-authorisation/availability-medicines

[7] Directive 2011/24/EU of the European Parliament and of the Council of 9 March 2011 on the application of patients' rights in cross-border healthcare https://eur-lex.europa.eu/legal-content/EN/ALL/?uri=celex%3A32011L0024

- adopting **a more harmonised approach in market authorisation** among the EU Member States, to ensure consistency of rules and equal access to treatment options
- giving a **stronger voice to patients** in often non-transparent processes such as pricing and reimbursement
- **anticipating market failures, and tackling logistical disruptions in the supply chain**, for example via a communications channel through which companies inform EU and Member State authorities of potential disruptions in medicine supply and that this information is transparent to other stakeholders such as patients.
- accelerate the **introduction of ePrescriptions as the new normal** across Europe to help healthcare systems plan in advance their medicines stocks

## Problems accessing allergy, asthma and COPD medicines in case of shortages

Medicine shortages can have a great impact on patients living with complex chronic diseases such as allergy, asthma and COPD. The unpredictability of adverse disease events, coupled with the long-term treatment needs, can potentially have a life-threatening effect on patients, while significantly undermining their quality of life and imposing additional fear to treatment change and effectivity.

**Allergy** is standing out in this respect, in particular given its increasing prevalence in Europe and globally[8]. A new analysis shows there are major differences today in how **allergen immunotherapy (AIT) products** are being approved and used in European countries[9]. Especially in recent years, rearrangements in the drug market for allergy at the national level have significantly affected their availability. For example in Germany, many allergen products have disappeared as a result of novel regulations, while in the Netherlands similar problems have arisen due to the enforcement of the EU Directive 89/342/EEC and reimbursement issues[10].

## Access for EFA and its patient community

EFA understands access to healthcare in a holistic way covering the following 5 elements (the so-called 5As): Availability, Adequacy, Accessibility, Affordability, Appropriateness.

Often, decision-makers do not feel the urgency on access as the situation does not lead to fatal events, but patients report clear suffering and higher costs, due to uncontrolled disease, frequent hospitalisations and sick leave[11]. The reasons behind these appalling Patient Reported Outcomes (PROs) are also the lack of real alternatives and treatment choices when a medicine is not being effective for the patient.

Withdrawals also provoke access inequalities among patients. As our members in Switzerland report, a provider of a Scoop catheter system did not renew the CE certification in 2015, but the national

[8] European Academy of Allergy and Clinical Immunology: By 2025 more than 50% of all Europeans will suffer from allergy - Advocacy Manifesto, Tackling the Allergy Crisis in Europe - Concerted Policy Action Needed, 2015 https://www.eaaci.org/documents/EAACI_Advocacy_Manifesto.pdf

[9] V. Mahler, R. E. Esch, J. Kleine-Tebbe, G. Plunkett, S. Vieths, D. I. Bernstein, ''Understanding differences in allergen immunotherapy products and practices in North America and Europe'', March 2019, https://www.jacionline.org/article/S0091-6749(19)30121-6/abstract

[10] European Academy of Allergy and Clinical Immunology, ''Challenges in the implementation of EAACI guidelines on allergen immunotherapy: A global perspective on the regulation of allergen products'', August 2017, https://onlinelibrary.wiley.com/doi/10.1111/all.13266

[11] European Federation of Allergy and Airways Diseases Patients' Associations, Atopic Eczema, Itching for Life: Quality of Life and Costs for People with Atopic Eczema in Europe, 2018 https://www.efanet.org/images/2018/EN_-_Itching_for_life_Quality_of_Life_and_costs_for_people_with_severe_atopic_eczema_in_Europe_.pdf

regulatory agency authorised an exemption for the existing patients using the device. Since 2017, the exemption has become permanent, however only valid for newly diagnosed patients.

Meanwhile, asthma, allergy and COPD are typically associated with increased **prevalence of comorbidities**[12,13,14]. Often patients have to deal with co-existing chronic conditions such as respiratory and cardiovascular diseases, which raises the cost they have to bear for their treatment[15]. Our members report that in the real world, many are led to neglect certain medicines due to cumulative pricing, and end up skipping their base treatment, which is more expensive but less aggressive, and entails less secondary effects than relievers.

In this light, EFA urges the Commission to propose an EU Pharmaceutical strategy that effectively tackles access issues in a way that:

- promotes a **holistic approach on access** on the basis of 5A principles: Availability, Adequacy, Accessibility, Affordability, and Appropriateness
- takes into account **cumulative pricing in drugs treating documented comorbidities**, and potential consequences of this cost in their quality of life
- improves **access to personalised drugs and therapies** for the treatment of disease subtypes and low-prevalence diseases by the alignment of quality standards and the harmonisation of market authorisation processes.

## Innovation in early development and authorisation

### Promoting innovative research in the EU

With regards to the development of new medicines, EFA stresses the need for an adaptive legal framework that embraces technological solutions and innovation with a **cure-oriented and high-end pharmaceutical research approach.**

As a recent Lancet study demonstrates, respiratory disease was the third leading cause of death in 2017 and takes the lives of an estimated 650,000 people each year (7% of deaths from all causes)[16]. Of course, the impact of respiratory disease can span a lifetime, with asthma affecting children and many other conditions resulting in a loss of quality of life and days off work[17].

An analysis of European Commission R&D spending (through Framework Programme 7, 2007 to 2013), showed a mere 0.5% of the €6bn health research budget went to asthma and COPD (€30 million)[18].

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[12] X. Su, Y. Ren, M. Li, X. Zhao, L. Kong, J. Kang, ''Prevalence of Comorbidities in Asthma and Nonasthma Patients: A Meta-analysis'', Medicine, 2016 https://www.ncbi.nlm.nih.gov/pmc/articles/PMC4900697/

[13] T. Greulich, B. J. D. Weist, A. R. Koczulla, S. Janciauskiene, A. Klemmer, W. Lux, P. Alter, C. F. Vogelmeier, '' Prevalence of comorbidities in COPD patients by disease severity in a German population'', Respiratory Medicine, 2017 https://www.resmedjournal.com/article/S0954-6111(17)30346-3/fulltext

[14] C. Cingi, P. Gevaert, R. Mösges et al. Multi-morbidities of allergic rhinitis in adults: European Academy of Allergy and Clinical Immunology Task Force Report, 2017 https://doi.org/10.1186/s13601-017-0153-z

[15] Wenjia Chen, Larry D. Lynd, J. Mark FitzGerald, Carlo A. Marra, Robert Balshaw, Teresa To, Hamid Tavakoli, Mohsen Sadatsafavi, ''Excess medical costs in patients with asthma and the role of comorbidity'', European Respiratory Journal, 2016 https://erj.ersjournals.com/content/48/6/1584

[16] GBD Chronic Respiratory Disease Collaborators, The Lancet, 2020, https://www.thelancet.com/action/showPdf?pii=S2213-2600%2820%2930105-3

[17] European Federation of Allergy and Airways Diseases Patients' Associations, Active Asthma and COPD Patients Access Care Report, 2019 https://www.efanet.org/images/ShowLeadership/Report-Showleadership_FINAL.pdf

[18] N. Papadopoulos et al, ''Asthma research in Europe: a transformative agenda for innovation and competitiveness'', European Respiratory Journal, 2017 https://erj.ersjournals.com/content/49/5/1602294

This is alarmingly low, especially if assessed against the overwhelming evidence on the actual impact of the diseases, expressed through morbidity and mortality.

Meanwhile, at a time when private funding for respiratory conditions is scarce, an example of innovative research collaboration is the ongoing 3TR project on identification of the molecular mechanisms of non-response to treatments, relapses and remission in autoimmune, inflammatory, and allergic conditions, which looks into seven conditions (asthma and COPD among them) to define why certain patients do not respond to certain treatments. Funded by the Innovative Medicines Initiative (IMI), 3TR looks into shared pathways of response to treatment and disease progression[19].

A pharmaceutical strategy for Europe should **promote public-private collaborations with robust funding**, as much as research networks and partnerships between regulatory authorities, industry, academia and non-profits, giving a **central role to patient organisations**. One such initiative is the European Partnership for Health Innovation (working title: Innovative Health Initiative: IHI) which is currently under consideration aiming to replace IMI. According to its draft proposal, IHI will seek to enable the integration of cross-sectoral technologies in support of health prevention, diagnosis and treatment[20]. The partnership will aim to bring together a variety of actors in a broad health R&D strategic discussion, including from the academia, healthcare professionals, the pharmaceutical industry, Health Technology Assessment bodies, patients and citizens. In this sense, the new EU Pharmaceutical Strategy should drive the work undertaken by the future European Partnership for Health Innovation through **early stakeholder engagement** aiming at tangible societal impacts[21].

EFA therefore encourages the Commission to propose an EU Pharmaceutical strategy that promotes innovative research in the EU:

- to **address the increasing burden of non-adherence to treatment**, especially among respiratory disease patients
- to **develop treatment options for underserved disease areas and disease subtypes**, such as severe atopic eczema
- to **support public-private collaborations promoting health innovation with robust funding, giving patients a central role**

## Facilitating future medicine development

A new pharmaceutical strategy should guide the way for an authorisation framework enabling **the market authorisation of innovative medicines and therapies**, provided that they are efficient and safe. While the existing authorisation process regulates very well standard medicines, biological medicines and personalised treatments often fall out of the validation rules.

Today there are specific issues around the evaluation of efficacy and safety of biological/personalised treatments, in that they cannot go through the authorisation pathway as standard medicines. This is because some of them are ideally designed to address specific biological characteristics of a specific patient or group of patients. Under the current common practice, the producers seek to have

[19] 3TR project official website, https://3tr-imi.eu/

[20] European Commission, Draft proposal for a European Partnership under Horizon Europe, European Partnership for Health Innovation https://ec.europa.eu/info/sites/info/files/research_and_innovation/funding/documents/ec_rtd_he-partnerships-for-innovative-health.pdf

[21] Draft proposal for a European Partnership under Horizon Europe - European Partnership for Health Innovation July 2020: https://ec.europa.eu/info/sites/info/files/research_and_innovation/funding/documents/ec_rtd_he-partnerships-for-innovative-health.pdf

authorised only a very small number of products: the need for extensive standardisation for each allergenic extract or combination of extracts incurs high costs, given the lack of a sizeable market for the least common allergies. Unfortunately, this leaves many patients without treatment options, and deviates from the 'precision medicine' principle that is the current trend. The situation affects several types of immunotherapy treatments such as phage therapy* as well as skin prick tests, typically used to diagnose allergy[22].

Addressing the above standardisation challenges could assist in **making personalised medicine/treatment approach the 'new essential'**, advancing whole scientific research fields and expanding patient access to innovative medicines and therapies. An example is the ongoing research on phage therapy, which beyond profiling itself as a solution to antimicrobial resistance, it could also advance the current stalemate on the treatment for chronic inflammation.

Finally, EFA hopes to see the European Medicines Agency (EMA) assume an even greater role than it has today. As the actor embodying the centralised procedure in the authorisation of medicines and thus setting the prerequisites for their placing in the EU market, EMA should be further empowered towards actions that enable the development of innovative medicines and therapies. Meanwhile, EMA should be encouraged to continue and even strengthen the involvement of patient organisations in its work, in continuation with its positive track record to date.

Therefore a new EU Pharmaceutical Strategy should support innovative research by:

- adopting **a cure-oriented pharmaceutical research approach** based on earmarked public funding for research
- address the **discrepancy between promoting patient involvement in medicine development and the perception of conflict of interest** in the involvement in regulatory processes
- promote **medicine development that addresses the real needs of patients**
- driving **public-private partnerships bringing together patients, researchers and the industry** towards high-end under-researched therapeutic areas
- enhancing **transparency by publishing qualitative and quantitative data** on the impact of the diseases compared to what is spent for research purposes
- proposing a **friendlier framework enabling the marketisation of innovative medicines** and therapies
- **supporting disruptive science** in response to persistent health challenges such as antimicrobial resistance, opening the way to new drug development pathways
- **empower EMA in taking up EU-wide initiatives in the area of innovative medicines** and further engage with patient groups
- fully **acknowledging the current problem of non-homogeneous authorisation procedures** and promote the development of specific frameworks for biological/personalised treatments such as AIT and phage therapy, towards the direction of step-by-step approvals, based on safety/efficacy and product effectiveness

## Research and development in areas with limited or no therapeutic options

Allergy, asthma and COPD are multi-faceted chronic airways diseases, characterised by complex and interacting epigenetics, trigger factors, and severity. Despite COPD's high prevalence among the

[22] L. Klimek, H.J. Hoffmann, H. Renz, P. Demoly, T. Werfel, P.M. Matricardi, A. Muraro, P. Schmid-Grendelmeier, V. Cardona, N.G. Papadopoulos, 'Diagnostic test allergens used for in vivo diagnosis of allergic diseases are at risk: A European perspective', Allergy, 2015 https://bit.ly/2FBCvvF
*Phage therapy is a promising treatment which can help balance inflammation in the organs of people with allergy and asthma. In phage therapy, phage cocktails are continuously developed to respond to changing resistance of bacteria.

population, today there is no efficient treatment for COPD at all, which incurs a huge unmet need and burden for patients.

In fact, low-prevalence allergies is an area with limited or no targeted/effective treatment options at the moment. This is mainly because allergen immunotherapy is often linked with **difficulties to fulfil the recruitment and other requirements of clinical trials**, as well as commercial considerations. Policy fragmentation is a concern here too, as national reimbursement decisions influence the availability of marketed medicines and therapies.

Another example is the one-drug-fits-all concept applied to airways disease, that leaves many patients with high medication doses and risk for side effects, but little disease control. It is the case of asthma and allergy (currently impacting more than 150 million citizens in Europe[23]) which are divided into disease sub-types with lower prevalence but more pressing needs.

Research aspiring to pharmaceutical breakthroughs for allergy, asthma and COPD is crucial to be able to truly address the unmet needs associated with the diseases. At EFA we believe medicine development initiatives should be driven by quantitative and qualitative assessments such as the quantitative burden for the targeted patient community and the **benefits of a new medicine for patients' quality of life**.

In the recent past, EFA has encouraged the EU to seek regulatory convergence among EU Member States, for example through scientific guidelines on the development of allergen products to treat allergies, emphasising the need to enhance access to treatment so that no allergy patient is left behind. Accordingly, incentives should be provided for the development of **safer medicines with less secondary undesired effects**. According to the EFA Access report, half of asthma and COPD patients are concerned about the cumulative side effects of their treatment, and therefore consider research on new and safer therapies with fewer side effects as a top priority[24].

In relation to innovation in disease areas with limited or no therapeutic options, EFA calls on the European Commission to come up with a strategy that:

- Focuses on **identifying a common understanding on the areas of unmet need in the EU**, and a consensus on the definition of those areas reached with the participation of patient organisations
- Rationalises the areas where **drug development can bring high cost-benefit ratios**, such as underserved diseases like COPD
- **Streamlines standards for clinical trials for low-prevalent diseases**, while supporting the conduct of so-called "pragmatic trials" in cases where the number of available patients for a clinical trial in a controlled setting is low
- Supports **research on new and/or targeted treatments and therapies at the EU level**, taking advantage of advances in the areas of genomics and biomarkers

## Opportunities and risks of digital technologies

Patients are benefited by the digital revolution in the area of health, both as individuals and at the macro level. eHealth and mHealth offer a great potential for **patient empowerment and the modernisation of health systems** alike, and can facilitate **personalised medicine in care**. Moreover,

[23] European Academy of Allergy and Clinical Immunology, Advocacy Manifesto: Tackling the Allergy Crisis in Europe - Concerted Policy Action Needed, 2015 https://www.eaaci.org/documents/EAACI_Advocacy_Manifesto.pdf

[24] European Federation of Allergy and Airways Diseases Patients' Associations, Active Asthma and COPD Patients Access Care Report, 2019 https://www.efanet.org/images/ShowLeadership/Report-Showleadership_FINAL.pdf

digital tools can **improve disease and self-management patterns**, from diagnosis and adherence to therapy thanks to the use of Real World Evidence.

For example, **misdiagnosis** remains a major clinical concern in allergy, asthma and COPD. According to the EFA Active Patients Access report findings, 16% of asthma patients get a wrong first diagnosis, while this percentage is also holds true for COPD patients[25]. New and digitalized data can support researchers better identify **phenotypes** and underlying disease mechanisms, classifying bulk diseases such as asthma and therefore leading to more specific treatments and quality of life.

Furthermore, **digital solutions and applications are key enablers of disease self-management**, as they assist patients in accessing their health data and reducing restrictions based on their condition. Critically, digital health tools can help in recording and monitoring the disease action plan, where applicable. As the above EFA report demonstrated, written management plans are extremely useful for patients, enabling them to react to asthma or COPD exacerbations and emergencies, prevent risk factors and integrate physical activities. Therefore, the EU should support digitally-enabled self-management of disease as part of medicines and other treatment methods.

Considering the long-term treatment requirements of chronic conditions such as allergy, asthma and COPD, EFA supports every initiative that reinforces the cross-border exchange of data in the context of existing structures such as the **Electronic Health Records and ePrescriptions**. In addition, public health **education and digital literacy** need considerable attention, as they represent key elements of patient empowerment. Of particular relevance here are diseases such as COPD, which typically affects the elderly. Through targeted actions, all age groups should be offered the possibility to join the digital transition that submerges our societies.

The promise of digital technologies cannot be viable if patients are not at the centre of disease management, data collection and digital use. Therefore, at EFA we believe that the EU approach towards digital health, including in the context of the announced common EU health data space, should be based on rules that preserve patients' ownership of and access to their data, clarity on how data are collected and used, as well as the portability/interoperability of data within and across countries, and among different e-applications.

Thousands of patients enrol in clinical trials voluntarily every year in Europe and that can be expanded with digital health. However, patients' data should not become a commodity to serve commercial interests[26], but rather the centre-piece for the development of treatment breakthroughs.

Finally, today we are witnesses in a revolution in digital healthcare, embodied by the increasing use of Artificial Intelligence and Big Data. However, for all their undisputed benefits, not least in diagnostics, care delivery and self-management, one should not disregard potential challenges. For example, as the European Patients Forum has pointed out in its recent contribution to the Artificial Intelligence White Paper, giving full diagnostic power to AI systems bears the **risk of overdiagnosis and overtreatment**, therefore increasing unnecessarily the number of medical interventions and the potential harm to patients.

To fully benefit from the opportunities brought by the digital transition, the future pharmaceutical strategy for Europe should:

- **Respect patients and their data-sharing** as cornerstone of medicines development and

[25] European Federation of Allergy and Airways Diseases Patients' Associations, Active Asthma and COPD Patients Access Care Report, 2019 https://www.efanet.org/images/ShowLeadership/Report-Showleadership_FINAL.pdf

[26] Charter of Fundamental Rights of the European Union, 2012 https://eur-lex.europa.eu/legal-content/EN/TXT/PDF/?uri=CELEX:12012P/TXT&from=EN

assessment, by enabling their involvement and engagement in decisions about digital health
- **Consider adherence and self-management guidance** as an integral part of the strategy with the support of Real World Evidence
- Serve the **improvement of health and digital literacy, as well as** public health education

### Towards clinical trials that incorporate patients' needs and perspectives

Clinical trials are the baseline for the development of efficient and safe medicines. At EFA we have long advocated for the increased involvement of patients in the clinical trials process, greater transparency for clinical data results, and strengthening the connection between clinical trials and real-world health effects[27][28].

EFA firmly maintains that clinical trials should be designed in a way that ensure the **involvement of patients since the early stages of the medicine development**. Equipped with their real-world and, above all, first-hand experience from living with the diseases, patients should participate in the **setting of the research agenda** and the conduction of the clinical studies.

The empowerment aspects of patients' participation in the clinical trials process are also illustrated by other factors, especially linked to the **readability and dissemination of clinical trials results**. Having a clear and understandable summary of the results is critical, as it ensures the timely flow of information to patients in lay language. On this basis, patients may also decide on future involvement in other clinical trials and they will get up-to-date information on the latest treatment progress and scientific breakthrough on their disease areas.

Further, as mentioned above, we strongly support optimising treatment to patients through **supporting the conduct of so-called "pragmatic trials"**. Pragmatic trials have the power to complement explanatory trials with evidence that are often easier to be found in the real world than in the lab e.g. adherence to a treatment. This can be especially useful in the case of low-prevalence diseases, where the recruitment of an adequate number of patients for the conduct of clinical trial in a controlled setting is difficult or impossible.

At EFA, we call on the Commission to integrate a broader approach to clinical trials, including by:

- Ensuring the **involvement of and engagement with patients** since the early stages of medicines development
- The **facilitation of pragmatic trials** that can offer significant real world insights into diseases of low prevalence

## Environmental sustainability of medicines and health challenges

Certainly, the manufacturing and use of medicines and medical equipment has an environmental footprint on the planet, with potential health implications. Instead of saving lives and helping treat diseases, medicine residues and disposed medical devices may end up polluting the soil, water and air, posing a threat to the ecosystem.

Treatment choices can and should be improved to meet environmental requirements. As patients' representatives, EFA holds that the advancement of health and the environment can and should go hand-in hand. To this end, we have promoted the avoidance of unnecessary waste when using drug-

[27] European Federation of Allergy and Airways Diseases Patients' Associations, Clinical Trials https://www.efanet.org/care/healthcare/clinical-trials

[28] European Federation of Allergy and Airways Diseases Patients' Associations, response to the European Commission open consultation on the summary of results of clinical trials for laypersons, 2016 https://www.efanet.org/news/3052-simplified-clinical-trials-results-to-inform-patients-our-response-to-the-ec-consultation

device combinations, through the separate commercialisation of canisters as well as information on the recyclability and carbon footprint impact of the device[29].

Besides, EFA is concerned about the potential outcomes of asthma and COPD care in Europe, if human health and care is not part of the evaluation on F-gases. In our recent contribution to the F-Gases regulation consultation, EFA argues that environmental sustainability should still be achieved while taking on board health management considerations such as the healthcare professional's knowledge, the patient's inhalation capacity, the disease severity, the patient's ability to use their device correctly, and the patient's personal preference.

EFA therefore encourages the Commission to promote in the pharmaceutical strategy the following:

- the **development of medicines and medical devices that are green, smart, and recyclable**

## Use of medicines and the fight against AMR

EFA has always been supportive of the European Patients Forum in its efforts to raise awareness on antimicrobial resistance, a topic that can potentially affect the lives of millions of people. Of course, safe use of medicines is key for all patient, regardless of age or disease. From the perspective of allergy, asthma and COPD, patients need to be more empowered to avoid unnecessary antibiotic use, as they need it for their serious exacerbations which, unfortunately, are part of their life with a chronic respiratory condition. This said, we fully agree with the EPF position that for any restrictions exemptions should be in place to ensure patient's medical needs are met.

## COVID-19 pandemic and access to treatment and medicines

The SARS-CoV-2 pandemic is transforming the global health landscape. On the one hand, healthcare systems have transformed their care flow to respond to the pandemic. On the other, well working services have collapsed.

Access to treatment and medicines has not been an exception: the health crisis exposed the EU's shortages in certain medical material and equipment, especially in terms of active pharmaceutical ingredients. Part of the problem has been the over-dependence on imports from markets outside the EU, where severe disruptions in the supply chain took place as a consequence of the COVID-19 emergency[30].

A common denominator for chronic respiratory patients across the EU has been the deterioration in the access to care services. With a closer look, and following discussions with several of EFA members, other country-level details and trends also emerged: for example, access to healthcare during the COVID-19 crisis proved particularly challenging in countries with a federal system, with stark differences among regions or even among hospitals in the delivery of care (Italy, Spain, Czech Republic). This resulted in serious delays in treatment, long recovery times, or even total lack of access to specialist care.

In some cases respiratory patients also had to deal with medicine shortages, as a result of disrupted supply or because critical medicine was used for COVID-19 patients (Italy and Czech Republic, respectively). In Switzerland, the crisis revealed issues with hospital supply and ambulatory care for oxygen, while sparking discussions on who would be delivered oxygen first.

Over time, the expansion of digital/phone consultations helped balance the situation in some of the cases (Italy, Czech Republic). In the latter home care was supplemented by the extensive use of ePrescriptions, which provided further support to patients for the management of their disease. Given

[29] EFA response to EMA public consultation on Drug-Device Combination, 2019
https://www.efanet.org/news/3757-efa-response-to-ema-public-consultation-on-drug-device-combination
[30] European Commission, https://ec.europa.eu/health/human-use/strategy_en

that in a normal situation people with asthma and COPD visit the doctor several times per year (1 in 3 asthma patients see their doctor from 6 to 11 times per year, while more than half of COPD patients see their doctor more than 6 times per year), it is positive to see other means that can ensure the continuity of care in times of emergency[31].

## Crisis preparedness and pharmaceuticals

Given that epidemiologists in their large majority do not expect a demise in Covid-19 outbreaks before an effective vaccine has emerged, it is necessary that the EU takes all **necessary steps to improve preparedness and coordination among Member States**. Therefore EFA believes that the Commission's intention, as stated in the recently proposed EU4Health budget, to enhance availability by creating an EU stockpile of medicines and medical devices, moves towards the right direction and responds to a real need of our times[32]. However, this stockpile should not only be limited to the current COVID-19 pandemic, but rather be applied in a rational way to treatments for non-communicable diseases that encounter regular shortages.

Learning from the Covid-19 emergency experience, the EU should have a role in helping coordinate preparedness and inform Member-States, and at the very least, prevent competition among them in times of crisis.

*Submitted by EFA with the input from expert patients and patient representatives from the EFA member-based Working Group on Allergy and Asthma, Working Group on COPD, Working Group on Atopic Eczema, Working Group on Food Allergy, and Working Group on Patient Education.*

[31] European Federation of Allergy and Airways Diseases Patients' Associations, Active Asthma and COPD Patients Access Care Report, 2019 https://www.efanet.org/images/ShowLeadership/Report-Showleadership_FINAL.pdf

[32] Proposal for a Regulation of the European Parliament and of the Council on the establishment of a Programme for the Union's action in the field of health for the period 2021-2027, https://eur-lex.europa.eu/legal-content/EN/TXT/?uri=CELEX:52020PC0405