RARE2030

# D5.4 RARE2030 Validated Scenarios

January 2021

## Report Information

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### Project Information

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# EXECUTIVE SUMMARY

This report aims to consolidate and liaise the qualitative RARE2030 storylines, contained in D5.2, with two subsequent project activities and one – external – extraordinary event that has profoundly changed the landscape on which the RARE2030 Scenarios were imagined.

The first section is dedicated to represent how the Scenarios were used in the policy work carried out by WP6 to devise the RARE2030 policy recommendations. In this light, we present a summary of the processes and outcomes of two main stakeholders' consultations: the Young Citizens Conference and the European References Network workshops. While this report primarily focuses on the modus operandi of the participatory process, Deliverable 2.4 and Deliverable 6.3 (Part 2) contain a detailed description of these two events results.

The outputs of 3 additional consultations allowed for the validation of potential scenarios and backcasting from preferred scenarios to policy recommendations. These include:

- 4 regional Rare 2030 conferences in Spain, France, Croatia and Italy and are described in Deliverable 6.3 (Part 1). This report highlight the needs for a number of EU level policy actions to make sure we do not find ourselves in Scenario 3 – "Its Up to You to Get What You Need" where *"health and social welfare systems are well integrated and patients may get the holistic care they need, but it will highly depend on the country in which they live".*
- 16 topic specific teleconferences across all recommendation topics with the Rare 2030 Panel of Experts the summaries of which are reported in Deliverable 6.2
- The Rare Barometer Survey with 3663 responses from people living with rare diseases across Europe reported in D6.1

Policy impacts of long-term scenarios can only be assessed and evaluated after sufficient time has elapsed for these impact to materialize. However, it is possible to reflect on the extent to which the process of building policy scenarios and recommendations has been able to promote understanding among experts and to involve stakeholders in the identification of policy options. This is indeed a 'secondary' goal of almost of all foresight processes triggered by the increased specialisation of experts and the emergence in the policy sphere of new actors, processes, metrics and ways of communication. In this light, the events summary aim to highlight as these back-casting workshops have been proven successful as an innovative way to create bridges between technical fields, to jointly establish priorities at medium and long term, and connect them to the overall European policy landscape.

The second section of this report describes the challenges identified, the responses formulated and the policy discussion emerged in the last year following the COVID-19 crisis. Beyond the immediate policy reactions, some key documents recently published by EU institutions and research organisations are reviewed in order to detect emerging risks and opportunities that could highly impact the future of health policy, health-systems organisation and rare diseases patients quality of life.

The third section briefly presents the Pharmaceutical strategy for Europe and evaluation of the legislation on rare diseases and children in order to contextualise the findings of the quantitative analysis reported in D5.3. The key findings of D5.3 are reported to inform the "Fast over Fair Scenario" Scenario – identified as the baseline scenario. The goals and priorities actions set by RARE2030 Recommendation "Available, Affordable and Available Treatments" are then included to highlight the pathway devised by stakeholders' consultation to move toward the most desirable "Investment for Social Justice" Scenario.

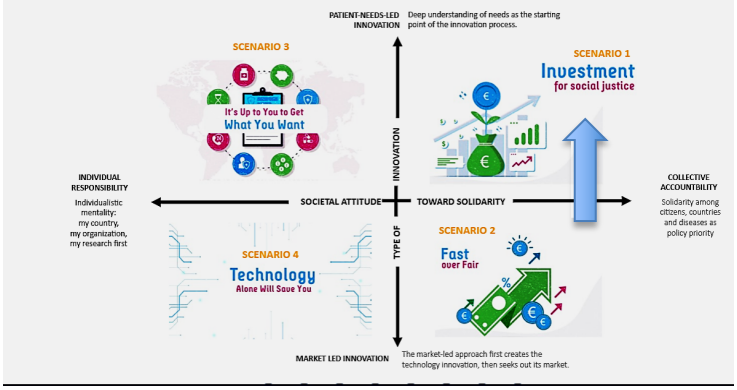



Figure 1. RARE 2030 Scenarios.

# 1. RARE 2030 From imagining Scenarios to a roadmap definition

## 1.1 Young citizens conference summary

In order to consolidate the RARE2030 scenarios (Annex 1), the consortium recruited and engaged young citizens in a series of online meetings, conferences and capacity building opportunities to elicit their opinion on the future of the rare disease policy.


*Figure 1 Rare 2030 Young Citizens*

The 28 Young Citizens (YC) coming from 12 European countries were recruited by EURORDIS through an online selection procedure which took into consideration: i) their nationality, ii) their fluency in English and iii) their age (between 18 and 32 years old). Pre-requisites for the selection procedure were a strong motivation and interest in rare diseases and in one of the following fields of expertise and/or background: patient representatives, advocate members, siblings of people affected with rare diseases, students, health professionals, health policy, public health, health economics, human rights experts or rare disease patient advocates themselves. More information of YC profiles is available at: https://www.rare2030.eu/who-is-involved/young-citizens/

Since the pandemic did not allow to organize a physical event, the Young Citizens Conference was devised as a sequence of different consultation opportunities including webinars, the European Conference on Rare Diseases and Orphan Products (ECRD), the Young Citizens Conference and the Young Citizens Fall Debate.

Specifically, young citizens were invited to attend, between March and May 2020, three webinars and the ECRD in order to provide inputs and ideas on the RARE 2030 scenarios.On the 7th and 8th of July, the Young Citizens Conference (YCC) was organized involving young citizens and 10 experts. The YCC focused on the following 8 discussion topics , derived from the knowledge-based documents produced in the first step of the RARE2030 project and analyzed through the lens of the RARE 2030 scenarios:

1. Political & strategic frameworks relevant to rare diseases
2. Data Collection and Utilisation
3. Availability and accessibility of Orphan Medical Products (OMPs) and medical devices
4. Basic, Clinical, Translational and Social Research for Rare Diseases
5. Diagnostics
6. Integrated, Social and Holistic Care for People with Rare Diseases
7. Rare Disease Patient Partnerships

8. Access to Healthcare

In line with the feedback collected during the ECRD, young citizens involved in the YCC also identified the "Investment for Social Justice" scenario as the preferred one, "Fast over Fair" as the most likely to happen while the "Technology alone will save you" Wasa ranked lowest

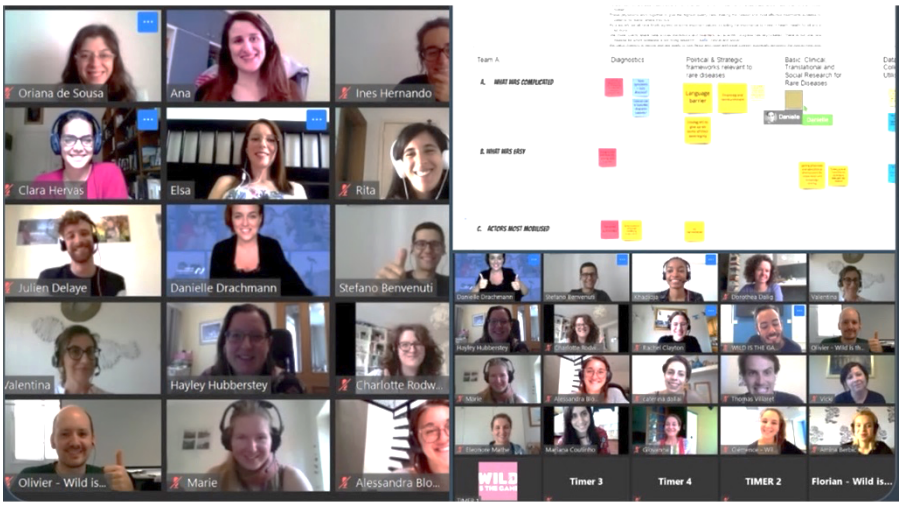



*Figure 2 YC Workshop*

Moreover, the participants elaborated a first draft of policy recommendations to reach the "Investment for Social Justice" scenario.

The Young Citizen Fall debate, held online the 28th of October, was the occasion for them to present their view on the future of people living with rare diseases (PLWRD) and advance policy recommendations proposals to a "jury" of high-level experts in the RD field. Divided in to 4 groups of 5/6 participants each, YC explained their vision and proposed the main recommendations to reach the "Innovation for social justice" scenario: an increased collaboration across Europe; a full involvement of all relevant stakeholders in research and care and the development of a holistic centric approach. Key steps to reach this vision are the provision of adequate education and training for all and a strengthening of digital health solutions. The table below provides a short overview of the elements characterizing the YC vision:

| | |
|---|---|
| **EU** | EU plays a central role in research, funding, HTA and access to integrated and person-centred care |
| **MS** | Member states collaborate together finding joint and sustainable solutions to ensure equal care as cross-border healthcare and joint procurement of medicines |
| **PLWRD** | • have access to current, clear, and thorough expert information on their disease<br>• have equal access to quick and accurate diagnosis and treatment irrelevant of location or socio-economic status (cross-border healthcare and reimbursement procedures)<br>• do not experiment discrimination<br>• own their data |
| **Academia and healthcare practitioners** | • have the same access to reliable, ethical, patient-driven data<br>• move the knowledge not the patient<br>• adopt a patient-centred research and care<br>• take care of the safety of patients' data<br>• work with health economics to produce evidence and data on cost-effectiveness in RD |
| **Industries** | • undertake fruitful partnership with patients and other stakeholders (clinicians, researchers, policy-makers)<br>• guarantee the safety of data |

*Table 1 YC vision elements*

A summary of the main recommendations provided on key areas of RD policies is reported in the table below. A full report of the Conference is available in deliverable D2.4 European "consensus conference" report

| Area | Recommendation | Main actors |
|---|---|---|
| Political and strategic framework for rare diseases | • Clarify and reframe the role of ERNs<br>• Reframe what it means to live with a RD<br>• Stimulate and increase public – private partnership<br>• Establish a unique European Rare Disease agency | Policy makers |
| Availability and accessibility of orphan medical products (**OMPs) and medical devices** | • Develop a more cohesive and more transparent reimbursement process<br>• Fostering cross –border healthcare<br>• Reduce restrictions on which disease can be prescribed specific treatments<br>• Foster joint-HTA assessment (hopefully an EU-wide initiative) | Insurers (private and public)<br>Industry<br>Policy makers |
| Diagnosis and research | • Setting up a minimum standard of *hard to diagnose* RD to be included in national plans for new-born screening (NBS)<br>• Apply comparative research on national NBS approaches for best practice identification<br>• Develop more company-oriented incentives<br>• Stimulate coordination between ERNs and hospitals<br>• Provide funding programs which could also foster the use of registries at the EU level<br>• Stimulate networking and promote partnership between actors, particularly between Patient Organisations (PO) and academia | Academia<br>Healthcare practitioners<br>ERN<br>Industry |
| Integrated, social and holistic care for people living with a rare **disease** | • Enhance education on the importance of holistic research and care<br>• Including PLWRD in research to better identify all their needs<br>• Promote awareness and training addressing discrimination also in the healthcare sector<br>• Enact laws against discrimination and stigmatisation<br>• Funding hospitals with regards also to mental health and social care | PO<br>Academia<br>Policy makers |
| Rare disease patient partnerships | • Recognize the value of patient-centric research<br>• Funding programs that promote networking with other stakeholders<br>• Develop training for other stakeholders<br>• Establish bi-directional specialised department for partnership in industry and PO<br>• Develop legal framework for data ownership and protection<br>• Promote the role of the expert patient as ethically regulated, acknowledged and paid | Academia<br>Industry<br>PO<br>(Policy makers) |
| Access to healthcare | • Improve articulation between public and private sectors<br>• Develop educational programs for healthcare professionals on patient's rights and cross-border healthcare<br>• Increasing the n. of centres of expertise<br>• Implement telemedicine<br>• Develop mixed funds scheme and practices for patients who need treatment abroad<br>• Facilitate authorization process and improving social support to patients while seeking care abroad. | ERN<br>Healthcare practitioners<br>Policy makers |

*Table 2 Young citizens policy recommendations*

## 1.2 ERNs Workshops – summary

Considering the pivotal role played by the European Reference Networks in RD research and specialized care, EU-level backcasting workshops were organized during September and October 2020, under the leadership of Victoria Hedley – University of Newcastle/UNEW – to reflect on future goals and challenges facing the ERNs. .
In lieu of organizing a single physical event as originally planned, the COVID-19 crisis compelled the RARE2030 team to hold four 2.5-hour on-line sessions (workshops) focused on specific topics (see Figure 5) and staggered across several weeks (21st- 28th- 29th September and 12th October), followed by a closing plenary on 26th October to present the main conclusions to all participants of the four specific sessions.

In preparing the workshops, the RARE2030 team devoted special attention to three key aspects:

- to ensure a balanced representation of all relevant stakeholders per group and per country. The overall process involved a wide number of participants, among which ERN coordinators, HCPs, patient advocates (ePAGs) and key representatives of European institutions;
- to ensure interactivity, engaging participants and allowing them to speak and contribute as in 'live workshops'. To this end, the company "Wild is the Game" was hired to guarantee technical support and provide expertise for creating a lively on-line experience. The realisation of *ad hoc* videos on the use of Zoom and Miro board – forwarded to the stakeholders before the events – contributed to ease the rolling out of the on-line format;
- to build on the findings of the Panel of Experts consultation (June-July 2020) aiming at gathering consensus on priorities and actions at different time horizons rather than at generating new knowledge. The sessions, the survey and the plenary setting and activities – shortly described below – were all adapted to support the participants' strategic thinking beyond the scope and mission of specific organizations and their field of expertise.

Each of the four workshops involved approx. 40 participants and focused on few key questions in each of the four areas of major strategic interest to ERNs. Strategic areas and key questions are reported in the table below.

| Strategic areas/ Session | Key questions |
|---|---|
| 1. Governance and Strategic positioning of ERNs | a. Should the ERNs have a legal status, and if so, what is the best route/best way to achieve this?<br>b. How can we secure financial sustainability of ERNs? (national support, European support, other avenues for funding)<br>c. Future composition of ERNs (in terms of model and scale): can we reach the right balance between Centres directly and indirectly involved in the ERNs? How should ERNs collaborate with stakeholders and countries externally (outside of the EEA – if indeed they should)<br>d. Planning ERNs Operational coverage and disease expansion (new) |
| 2. Integrating ERNs to national systems and frameworks | a. What is the best way to integrate ERNs into national health systems?<br>b. How should ERNs complement the wider national landscape of Centres of Expertise for RD? How should HCPs and 'affiliated' partners sit within the national ecosystems?<br>c. What role do you see ERNs playing in bridging health and social care?<br>d. Should ERNs drive future policies at national level? And if so, how? |

| | |
|---|---|
| 3. Role of ERNs in virtual care delivery and cross-border healthcare | a. How can the CPMS transform virtual care for specialized conditions, and how can the ERNs more widely accelerate positive telemedicine trends towards balanced physical-virtual clinics of the future?<br>b. What does success look like for you? Is it improving the health-related outcomes for patients visiting member HCPs or for whole populations? How do we achieve this?<br>c. How can we receive legal/regulatory/financial recognition of time and expertise spent on cross-site CPMS case discussions? Could/should decisions of ERN panels bear more weight (e.g., in terms of influencing decision-making on patients accessing treatments domestically or abroad?) |
| 4. ERNs, research, and the data ecosystem of the future | a. What should be the ERNs' 'data strategy' in 2030? How do we review the policies (and address the problems) around data-sharing and health & research?<br>b. How can ERNs contribute to diagnostic equality across Europe? What is the best, most realistic role for them to play?<br>c. How do we want ERNs to be positioned, research-wise?[1] Is the research side supported strongly enough by all countries and actors? How should ERNs engage with Industry in future? |

*Table 3 ERN EU Level Back-casting 'strategic areas' Sessions: areas and key questions*

Different in scope, the four sessions followed a similar agenda, comprising both individual work and break-out rooms, structured as follows:

| | |
|---|---|
| Welcome and short presentation | Yann Le Cam (EURORDIS) and Victoria Hedley (UNEW) provided a quick overview of the RARE2030 project and explained how the workshop complemented the objectives of the project and -importantly- the broader ecosystem |
| Digital icebreaker | Olivier Percevaut (Wild is the Game) demonstrated the use of Miro as platform working space |
| Individual work | Based on the findings gathered on the Panel of Experts consultation and presented in the Miro platform by the project team, the participants were invited to explore, comment and vote the most interesting responses to the proposed workshop questions (see table below) |
| Group Working: developing or discussing potential ERN-focused recommendations | The participants, split into groups of approx. 7 people, worked together to address one specific workshop question. The four breakout rooms – moderated by a facilitator and supported by a note-taker – were organised to further develop the draft recommendations explored above, to identify areas of consensus and disagreement and capture comments on 'implementation gaps' |
| Reporting back | The full group came back together and rapporteurs for each group briefly reported on some of their main conclusions and any recommendations they were able to already propose/support |
| Quick satisfaction poll and next steps | Feedback was elicited to help improving the next working sessions |

*Table 4 ERN EU Level Back-casting 'strategic areas' Sessions: agenda and settings*

[1] *'How do we secure funding for RCTs that create evidence for essential care/treatments of RD such as rehabilitation, which are neither industry-linked nor will likely attract research-funding at present?'*

Policy impacts of long-term scenarios can only be assessed and evaluated after sufficient time has elapsed for these impact to materialize. However, it is possible to reflect on the extent to which the process of building policy scenarios and recommendations has been able to promote understanding among experts and to involve stakeholders in the identification of policy options. This is indeed a 'secondary' goal of almost of all foresight processes triggered by the increased specialisation of experts and the emergence in the policy sphere of new actors, processes, metrics and ways of communication. In this light, the back-casting on-line workshops described above should be considered as an innovative way to create bridges between technical fields, to jointly establish priorities at medium and long term, and connect them to the overall European policy landscape. In line with this, in the break-out rooms, participants were invited to consider different time horizons (short, medium, long) and adopt a multi-governance perspective (local, national, European, global) while evaluating the feasibility (difficult/easy) as well as the impact (low/high) of the different policy options.


*Figure 3 ERN EU Level Back-casting Workshops worktable example (Miro visual platform screenshot).*

Through this process, the 80 recommendations - originally contained in the Miro Board as findings from the Panel of Experts calls – were condensed into 34 final items distributed across the four strategic areas. This was notably made possible by the collaborative effort of participants in discerning long-term goals from intermediary steps , thus helping to identify, and possibly merge, actions that are considered to be strictly interrelated.

In preparation of the closing ERN plenary on 26th October, a survey was launched to give all the attendees the opportunity to review the strategic area session results and rank the 34 recommendations by importance and impact. Taking the strategic importance of ERNs into consideration, it was decided to extend the consultation to external experts who were not involved in the four sessions. Ultimately, the survey gathered inputs whose contributions will inform the RARE2030 recommendations on the future of ERNs to be presented at the RARE2030 final conference on 23rd February 2021.

The closing event was structured so as to present the overall findings of the previous sessions along with the outcome of the strategic areas and then give the opportunity to representatives of the key actors in the field to comment from their specific point of view. The panel discussion involved representatives from ERN coordination, ePAG, the Board of Member States as well as from different DGs of the European Commission. Yann Le Cam, CEO of EURORDIS, opened the conference and the EURORDIS team supported the interaction with the attendees moderating the two Q&A sessions. Representatives from the European

Parliament, the European Commission, WHO and Orphanet contributed keynote speeches to set the ERNs vision in the framework of the future of global health and healthcare.

The Plenary proved highly successful, with the participation of 80 people of whom 16 ePAG representatives, 21 ERN coordinator/ERN Healthcare Provider representatives, 13 Board of Member States representatives, 4 ERN hospital managers, 8 European Commission representatives, 18 from EURORDIS and RARE2030 partners.

| ERN Plenary Event (online)<br>AGENDA<br>26th October 2020 - 9.00-12.00 CEST | |
|---|---|
| 9:00 - 9.05 | Welcome<br>Yann Le Cam, EURORDIS – Rare Diseases Europe |
| 9.05 - **9.20** | *Opening Session: Future Vision of ERNs: Perspectives from the European Commission, Parliament Members, and Board of Member States of ERNs*<br>Andrzej Rys, EC; Katerina Konecna, MEP; Till Voigtlander, BoMs |
| 9.20 - **9.40** | *Presentation of the key discussions, survey results and most preferred recommendations*<br>Victoria Hedley, UNEW |
| 9.40 - **10.15** | *Panel discussion and reactions:*<br>Perspectives from Nicoline Hoogerbrugge (ERN Coordinator), Birute Tumiene (BoMS), Rita Magenheim (ePAG Advocate) and Martin Dorazil (EC)<br>Moderator: Luca Sangiorgi, ERN BOND |
| 10.15 – 10.35 | Audience Interaction --- with Matt Bolz-Johnson, EURORDIS – Rare Diseases Europe |
| 10.35 - **10.55** | *Presentation of the key discussions, survey results and most preferred recommendations - From Session 3, Role of ERNs in virtual care delivery and cross-border healthcare; and Session 4, ERNs, research, and the data ecosystem of the future*<br>Victoria Hedley, UNEW |
| 10.55 - **11.30** | *Panel discussion and reactions*<br>Perspectives from Franz Schaefer (ERN Coordinator), Rebecca Tvedt Skarberg (ePAG Advocate), Gyorgy Pfliegler (BoMS) and Ioana-Maria Gligor (EC)<br>Moderator: Maurizio Scarpa, MetabERN |
| 11.30 – 11.40 | Audience Interaction--- with Ines Hernando, EURORDIS – Rare Diseases Europe |
| 11.40 - **12.00** | *Closing remarks: Perspectives from the Rare Disease Communities*<br>Key Opinion Leaders: Vytenis Andriukaitis, WHO Special Envoy for the European Region; Ana Rath, Orphanet; Enrique Terol, EC DG SANTE |


*Figure 4 ERN Plenary Event. Panel discussion.*

# 2. A Rapid changing landscape: risks and opportunities emerging from the COVID 19 crisis

The second section describes the challenges identified, the responses formulated and the policy discussion emerged in the last year following the COVID-19 crisis. Beyond the immediate policy reactions, some key documents recently published by EU institutions and research organisations are reviewed in order to detect emerging risks and opportunities that could highly impact the future of health policy, health-systems organisation and rare diseases patients quality of life.

A key political lesson of this crisis is that further collaboration is required in Europe to face health challenges such as the COVID-19 pandemic. Despite having little space for manoeuvre, the past European Commission mandate made some significant steps forward in terms of health policy. However, more efforts are needed, since Europe must tackle unprecedented challenges that increasingly require the implementation of a multi-sectoral, holistic and comprehensive approach to health. This is strongly advocated by RARE2030 policy recommendations and experiences and the knowledge of Rare Diseases communities in this can be a leading example and support the realisation of rapid and effective change from which will benefit PLWR as well as all European citizens.

## 2.1 EU Institutional response to the health threat

This section summarizes the challenges identified, the responses formulated and the policy discussion emerged in the last year following the health crisis and its uncertain future consequences.

Although the EU has developed a coordinated response to the coronavirus outbreak, calls have been issued for a comprehensive review of what went wrong– both in the Member States and at EU level – and for "drastically improving" preparedness[2]. In the EU, health care is a MS prerogative and in many EU countries regional and/or local authorities are mostly or partially responsible for the provision, funding and management of health systems. When confronted with such cross-border health threat, the multi-level governance framework has shown its flaws, as the effective and efficient crisis management called for rapid harmonisation and coordinated action superseding national borders.

The framework for EU response is the Cross-border Health Threats Decision (No. 1082/2013/EU) encompasses mechanisms such as information exchange, risk assessment and joint procurement, and assigns a key role to the European Centre for Disease Prevention and Control (ECDC) in identifying, assessing and communicating threats to health[3]. In spite of having a legally binding instrument and a dedicated agency, the EU governance framework for health crises remains a work in progress[4]: significant gaps persist when it comes to the implementation of the decision[5] and the EU framework is strongly constrained by the need to

[2] European Parliament (2020)- Coordinated by Franck Debié, Director for Library and Knowledge Services, DG EPRS; Member of the Steering Group of the European Strategy and Policy Analysis System (ESPAS); "Towards a more resilient Europe post-coronavirus An initial mapping of structural risks facing the EU"

[3] A 2019 report by the Global Preparedness Monitoring Board, published under the auspices of the World Health Organization, makes a compelling case for preparedness

[4] Andrea RENDA and Rosa CASTRO "Towards Stronger EU Governance of Health Threats after the COVID-19 Pandemia" Eur J Risk Regul. 2020 Apr 9 : 1–10. Published online 2020 Apr 9. doi: 10.1017/err.2020.34 PMCID: PMC7174850

[5] European Court of Auditors special report no 28, Dealing with Serious Cross-Border Threats to Health in the EU: Important Steps Taken but More needs to be Done (Luxembourg, Publications Office of the European Union 2016)

respect the competences of EU Member States[6]. The main coordinating agency – the ECDC – is also understaffed and under-budgeted.

The crisis has also revealed marked differences in the ability to cope with health emergencies across European national health systems. Hospital capacity, for example, varies greatly between EU countries and has strongly decreased in the last decades. Between 2000 and 2015, the number of acute care hospital beds per 100,000 population in EU registered an average reduction of 20.5%, more marked in Latvia (-44.6%), Denmark (-42.3%), Estonia (-38.7%) and Italy (-37.4%).[7] This reduction has probably contributed to the strain on healthcare systems during the coronavirus pandemic. The immense pressure faced by hospitals and healthcare workforce, combined with the lack of adequate preparedness to switch swiftly to new methods of service delivery (e.g., telemedicine, tele-monitoring and other e-health solutions)[8], has led to disruptions of prevention and continuity of care (notably in the treatment of cancer and other chronic diseases).

The crisis impact on the global supply chain and the subsequent shortage of personal protective equipment, medical devices and testing supplies has exposed EU's dependency on third countries in the health sector, prompting calls to relocate the production of essential medical goods to Europe[9]. In effect, 40% of medicinal and products marketed in the EU originate in third countries and 80% of active pharmaceutical ingredients are produced in China and India[10].

*To be able to better cope with future health emergencies and to drastically improve preparedness, EU and Member States need to build more resilient health infrastructure to deal with unforeseen events[11], and to raise the level of global preparedness making a more fundamental investment in health.*

The EU has recognized the weakness of the first phases of pandemic response[12], and has increased its efforts to create a common strategy against COVID-19 by catalyzing funding and capacities. EU's institutional response has been mainly led by the European Commission, and European Council members. The European Parliament and European Central Bank have also played important roles. In particular, in April 2020 an EP resolution on EU coordinated action against COVID-19 called for "new and strengthened instruments" so that, in future, the EU can coordinate "without delay" an emergency response, for instance, by "substantially strengthening" the European Centre for Disease Prevention and Control and the European Medicines Agency. The Parliament's call led the Commission to propose a new Health programme, EU4Health, which aims to strengthen Europe's health systems to respond better to future major cross-border health crises. The EU public health response mainly involves:

- direct financial support for procurement programmes to support healthcare systems;
- support for research into treatments and vaccines;
- medical guidance for Member States;
- coordinate the supply and manufacturing of Personal Protective Equipment (PPE).

[6] Anderson M, McKee M and Mossialos E, "Covid-19 Exposes Weaknesses in European Response to Outbreaks" (2020) 368 BMJ m1075 [PubMed].
[7] Pascal Garel and Isabella Notarangelo. Hospitals in Europe: Healthcare data. 9 January 2020. Available at: https://hospitalhealthcare.com/latest-issue-2018/hope-2018/hospitals-in-europe-healthcare-data-9/
[8] EC. Opinion of The Expert Panel On Effective Ways Of Investing In Health in the Organisation of resilient health and social care following the covid-19 pandemic. November 2020.
[9] European Parliament. Towards a more resilient Europe post-coronavirus. An initial mapping of structural risks facing the EU. July 2020.
[10] European Parliament. Committee on the Environment, Public Health and Food Safety. REPORT on the shortage of medicines – how to address an emerging problem. 22 July 2020.
[11] European Commission. Joint European Roadmap for lifting coronavirus- containment measures. 2020.
[12] European Parliament. Towards a more resilient Europe post-coronavirus. An initial mapping of structural risks facing the EU. July 2020.

| Instruments | When | Areas |
|---|---|---|
| Early Warning and Response System | Immediate January 20 | EWRS offers the possibility to MS to send alerts about health events with potential impacts on the EU, to share information and to coordinate their responses. |
| ECDC | Immediate January 20 | ECDC provides rapid risk assessments, frequent epidemiological updates and guidance on how to best face the outbreak. |
| COVID-19 advisory Panel | March 20 | The Panel formulates science-based EU response guidelines and coordinates risk management measures. Among the Recommendations provided, the ones for community measures and testing strategies and Health Systems Resilience. |
| RescEU stockpile of medical equipment | March 20 | The RescEU is part of the European Civil Protection Mechanism which strengthens cooperation between participating states in the field of civil protection. The stockpile includes intensive care medical equipment (e.g., ventilators), personal protective equipment (e.g., reusable masks), vaccines, therapeutics and laboratory supplies. |
| Standards for certain medical devices and equipment | March 20 | European Committee for Standardisation and the European Committee for Electro-technical Standardisation have established a number of European standards for certain medical devices and personal protective equipment. Stardards are aimed to support the quick production and the easiest placement in the internal market while ensuring a high degree of safety. |
| Practical guidance for across border cooperation | April 20 | The EC drafted practical guidance to MS in order to ease cooperation across borders in transferring and treating COVID-19 patients. |
| Solidarity Fund and Emergency Support Instrument | April 20 | The Commission has extended the Solidarity Fund to cover public health emergencies and has approved the Emergency Support Instrument to directly support the Member States' healthcare systems in their fight against the pandemic. |
| Funding therapies and diagnostic tools | May 20 | Currently more than 660 million have been raised to finance the largest number of projects focused on the development of therapies and diagnostics for the SARS-CoV-2. |
| "Coronavirus Global Response" | May 20 | EC Initiative to support the WHO "Access to COVID-19 Tools Accelerator" (ACT-Accelerator), programme established to find the resources needed to reduce the time and cost of vaccines and testing. |
| European Investment Bank loans and financing agreement | May 20 | The Commission offered CureVac financial support through a €75 million loan guarantee from the European Investment Bank (EIB).<br>The EIB also signed a €100 million financing agreement with the immunotherapy company BioNTech SE for the development of a vaccine programme. |

*Table 5 European initiatives promoted as COVID-19 response*

## 2.2 The role of Health in the new Multiannual financial framework and Next-Generation EU

The EU has taken swift actions in response to the challenges identified. The European Commission has mobilized more than €660 million under Horizon 2020 since January 2020 to develop vaccines, new treatments, diagnostic tests and medical systems to prevent the spread of the coronavirus and save lives[13] (Fig. 6). Through the European Investment Bank, the EU has also further boosted the vaccine programme and is currently engaged in ensuring its equal supply in all EU countries.

[13] EC. Overview of the Commission's response. Last access: 31 December 2020.

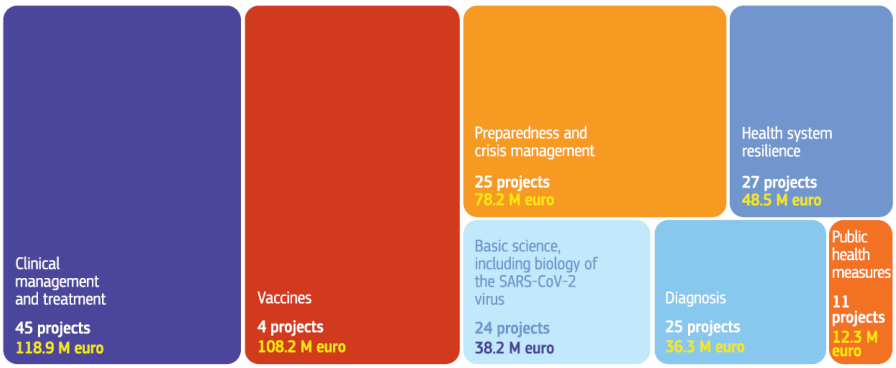



*Figure 6 Distribution of COVID-19 Horizon 2020 projects according to major needs by EU financial contribution (million euro). Updated in September 2020[14].*

The multiannual financial framework (MFF) for 2021-2027, the Next Generation EU and other EU initiatives and programmes are directed to strengthening cooperation, building capacities and resilience and enhancing the EU post-pandemic recovery. The EU long-term budget 2021-2027 (MFF) and the Next Generation EU, aimed at rebuilding a greener, more digital and more resilient Europe, form the largest stimulus package ever financed through the EU budget (€1.8 trillion, €1.074 trillion for MFF and € 750 billion for Next Generation EU). The funds raised will finance specific programmes such as: Recovery and Resilience Facility; REACT-EU; Rural development; Just Transition Fund; InvestEU; rescEU; Horizon Europe; Digital Health Programme; Erasmus + and many others. The table below provides a short overview of each initiative:

| EU4Health | Budget: €5.1 billion |
|---|---|
| EU4Health is the fourth and largest of the EU Health Programmes since its inception in 2003. Main aims are:<br>1. boost EU's preparedness for major cross border health threats by creating<br>• reserves of medical supplies for crise<br>• a reserve of healthcare staff and experts that can be mobilised to respond to crises across the EU<br>• increased surveillance of health threats<br>2. strengthen health systems so that they can face epidemics as well as long-term challenges by stimulating<br>• disease prevention and health promotion in an ageing population<br>• digital transformation of health system<br>• access to health care for vulnerable groups<br>3. make medicines and medical devices available and affordable, advocate the prudent and efficient use of antimicrobials as well as promote medical and pharmaceutical innovation and greener manufacturing[15]. | |
| The EU4Health defines the fight against cancer, reducing the number of antimicrobial-resistant infections and improving vaccination rates as health priorities to tackle.<br>The EU intends to continue to support successful initiatives like the European Reference Networks for rare diseases and to pursue international cooperation on global health threats and challenges.<br>After all, the EU Covid-19 clinical management support system (CMSS) launched on 24 March 2020[16] is based on the experience and know-how gained with the European reference networks (ERNs) – virtual platforms for voluntary cross-border collaboration between specialists in rare and complex diseases. The CMSS has been launched with the intent to create rapid connections across Europe among the MS reference hospitals for treating | |

[14] EC. EU research and innovation in action against the coronavirus: funding, results and impact. September 2020.
15 EC. EU4Health 2021-2027 – a vision for a healthier European Union. Available at: https://ec.europa.eu/health/funding/eu4health_en. Last access: 31 december 2020.
16 EC. Health and Food Safety Directorate General. Commission launches "COVID-19 Clinical Management Support System". 27 March 2020.

| | |
|---|---|
| Covid-19 patients. It could also serve as a basis for developing ERNs on rare and low-incidence infectious diseases, as proposed in a Commission feasibility study. | |
| **REACT-EU** | Budget: €47.5 billion |
| REACT – "Recovery Assistance for Cohesion and the Territories of Europe" is an initiative that in the cohesion policy framework continues the measures delivered through the Coronavirus Response Investment Initiative and the Coronavirus Response Investment Initiative Plus. The mechanism will aim to support crisis recovering and support green, digital, and growth-enhancing investment, in particular:<br>• supporting the reinforcement of crisis response capacities in health care;<br>• making a concrete difference in lives and jobs of people affected by the crisis, as the crisis repair measures will contribute to supporting job creation and maintenance, including through short-time work schemes and support for the self-employed, and providing urgent and much needed support directly to SMEs;<br>• enhancing support to infrastructure providing basic services to citizens[17]. | |
| REACT-EU provides a €47.5bn additional investment under the investment for growth and jobs goal. Member States can use the REACT-EU budget for the ESF, the European Regional Development Fund (including for cross–border co-operation under ETC), the Fund for European Aid to the Most Deprived (FEAD), and the Youth Employment Initiative and it will allow Member States and regions to finance health entities directly.<br>Exceptionally, the funding mechanism will be not broken down per region or sector and it will allow targeting the areas (geographical or sectoral) where support is most needed. In addition, it is ensured a high level of pre-financing (50%) and the possibility to finance additional amounts from the EU budget in order to ensure that a possible lack to match this with national co-financing is not an obstacle for the use of EU support. | |
| **Horizon Europe** – budget | Budget: €80.9 billion |
| The forthcoming EU programme for research and innovation, Horizon Europe is based on 3 pillars and on the intent to *Wide the Participation and Strengthening the European Research Area*. The three pillars are:<br>• Excellence science<br>• Global challenges and European Industrial Competitiveness<br>• Innovative Europe<br>Horizon Europe adopts the mission-oriented approach, identifying 6 strategic clusters, including health, and 5 missions namely cancer, adaptation to climate change including societal transformation, climate-neutral and smart cities, healthy oceans, seas, coastal and inland waters and soil health and food[18]. | |
| **RescEU** | Budget: up to €380 million for medical stockpile /up to €77 million for rescEU transition and capacities |
| In March 2020, the Commission created a rescEU strategic medical stockpile, hosted by one or several EU Member States, to enable swift distribution of medical equipment, such as ventilators, personal protective equipment, vaccines and therapeutics, and laboratory supplies[19]. RescEU, a part of the Union's civil protection mechanism that has the objective of enhancing both the protection of citizens from disasters and the management of emerging risks, will be expanded in view of future crises. | |
| **Digital Europe** | Budget: €7.5 billion |
| Part of the Multiannual Financial programme, Digital Europe is focused on building the strategic digital capacities of the EU and on facilitating the wide deployment of digital technologies. The programme will boost investments in supercomputing, artificial intelligence, cybersecurity, advanced digital skills, ensuring a wide use of digital technologies across the economy and society, including through Digital Innovation Hubs[20]. | |

*Table 6. Main EU initiatives to sustain the EU recovery and the resilience*

[17] EC. Cohesion policy at the centre of a green and digital recovery.2020. Available at: https://ec.europa.eu/regional_policy/sources/docgener/factsheet/2020_mff_reacteu_en.pdf
[18] EC. Horizon Europe. Available at: https://ec.europa.eu/info/horizon-europe_en Last access: 31 december 2020
[19] https://www.clustercollaboration.eu/news/european-commission-creates-first-ever-resceu-stockpile-medical-equipment
[20] https://ec.europa.eu/digital-single-market/en/europe-investing-digital-digital-europe-programme

## 2.3 Health: new risks on the horizon

In response to the coronavirus crisis, the European Union has devoted increasing efforts to anticipatory governance, notably through the analysis of medium- and long-term global trends, as well as through structured contingency planning and stress-testing of existing and future policies. This section reviews some key documents recently published by EU institutions and research organisations summarizing a preliminary analysis of the implications of the coronavirus pandemic for EU health policymaking. The questions we aim to address are "*how are health care systems and the health policy landscape changing following the coronavirus crisis? What might be the implications for the rare diseases community? Which synergies/risks can be foreseen with the recommendations designed to reach RARE2030 Scenarios?*"

In the aftermath of the COVID-19 outbreak (April 2020) the Vice-President of the European Commission invited the inter-institutional European Strategy and Policy Analysis System (ESPAS) to identify structural risks arising from the current pandemic with the view to help refine collective thinking on how to increase the long-term resilience of the Union over the coming decade. The EU Parliament (EP) paper "*Towards a more resilient Europe post-coronavirus*[21]" provides an initial "mapping" of 66 risks and outlines possible EU actions to prevent or mitigate those risks considered more relevant for the near future (2019-2024). Six out of the 66 risks identified were classified as specifically "social and health risks" – namely:

1. Health crises with pandemics of new infectious diseases or further disruptions due to the coronavirus pandemic,
2. Poverty and inequalities rising to unsustainable levels, including child poverty, housing issues and pension issues,
3. Gaps in the coverage of social protection systems,
4. Long term sustainability of social protection systems,
5. Failure to achieve gender equality,
6. Widening territorial divides and reduced cohesion.

The figure below provides a snapshot of whose selected risks evaluated by experts as more likely to happen and of generalized impact – those events, in fact, in need of 'immediate action'.

[21] European Parliament (2020)- Coordinated by Franck Debié, Director for Library and Knowledge Services, DG EPRS; Member of the Steering Group of the European Strategy and Policy Analysis System (ESPAS); "Towards a more resilient Europe post-coronavirus. A initial mapping of structural risks facing the EU". July 2020.

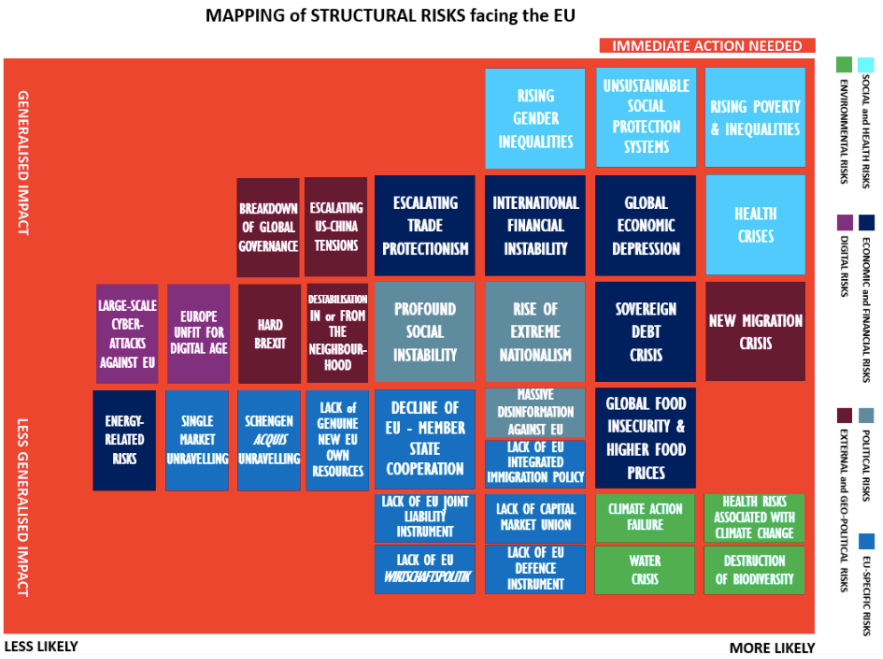



*Figure 7 Structural risks facing the EU. Source: EP. "Towards a more resilient Europe post-coronavirus. An initial mapping of structural risks facing the EU". July 2020.*

The EC "2020 *Strategic foresight report. Charting the course towards a more resilient Europe*"[2] (2020) aims to offer a comprehensive view of COVID-19 impact in order to ensure "*that short-term initiatives are grounded in a longer term perspective*"[22]. In this report, the central theme is how to build resilience and create sustainable transition in turbulent times in four key interrelated dimensions of EU societies: social and economic, geopolitical, green, and digital. The report recognizes resilience as the new compass for EU policies. "*Resilience is the ability not only to withstand and cope with challenges but also to undergo transitions in a sustainable, fair, and democratic manner*"[23].

The report starts with the analysis of the COVID-19 potential impact on the megatrends identified by the Joint Research Centre (see figure below), distinguishing the ones which are accelerating from those decelerating. Based on the analysis of risks and opportunities emerging from the crisis, the report takes into account the evolution of wellbeing, work, trading, labour markets and global value chains and charts EU role in promoting social, economic, digital and green transition.

[22] EC 2020 Strategic foresight report. Charting the course towards a more resilient Europe.2020.

[23] Manca, A.R., Benczur, P., and Giovannini, E., 2017, Building a scientific narrative towards a more resilient EU society. 23 Giovannini, E., Benczur, P., Campolongo, F., Cariboni, J., Manca, A.R, 2020. Time for transformative resilience: the COVID-19 emergency, Publications Office of the European Union, Luxembourg.


*Figure 8 Potential impact of COVID-19 on megatrends. Source: EC "2020 Strategic foresight report. Charting the course towards a more resilient Europe". 2020.*

In the Report, health issues are considered under the economic and social dimension and special attention is placed on the need to reduce inequalities, promote social and regional cohesion and support the most vulnerable in society[24]. The report underlines how the crisis has deepened inequalities, increased the number of citizens suffering from health and social vulnerabilities (e.g., persons with chronic diseases and disabilities) and raised the number of people in, or at risk of, poverty. In this regard, racial and ethnic minorities are statistically more at risk of facing financial insecurity. It also highlights that unequal access to digital infrastructure and services has widened the digital divide: lower skilled workers are more at risk to be employed in "contact jobs" and risk greater exposure to diseases whilst having lower access to healthcare. Women face a double burden as front-line workers and child-carers in lockdown period experiencing a significant increase of domestic violence[25].

As resilience has been identified as a new compass for EU policymaking, the report provides prototypes and preliminary "resilience dashboards" as monitor tools to indicate "the way to go" addressing pre-pandemic vulnerabilities and strengthening capabilities. Dashboards are based on a list of indicators drawn upon existing sectoral indicators and monitoring tools, - such as the *Social Scoreboard and the Monitoring report on progress towards the SDGs in an EU context*[26]– and intended to be dynamic and chosen through a participatory process involving MS and key stakeholders. The report contains a prototype dashboard for

[24] "The social and economic dimension of resilience refers to the ability to tackle economic shocks and achieve long-term structural change in a fair and inclusive way. It means building the social and economic conditions for a recovery geared towards the transitions, promoting social and regional cohesion, and supporting the most vulnerable in society, while taking into account demographic trends, and in line with the European Pillar of Social Rights".
[25] European Parliament. Towards a more resilient Europe post-coronavirus. An initial mapping of structural risks facing the EU. July 2020.
[26] https://ec.europa.eu/eurostat/web/products-statistical-books/-/KS-02-20-202

social and economic resilience linked to the COVID-19 Crisis (Figure 11) and for geopolitical, green, and digital dimensions of resilience. For each variable (indicator) included, dashboards highlight the relative situation of MS in the last year for which data is available versus pooled values of available data, across countries and years, since 2007. In this way, a global picture of vulnerabilities, resilience capacities, and common patterns throughout the EU is offered to suggest and pilot policy options.

Among other reports published on the pandemic impact, the World Economic Forum Report "*COVID-19 Risks Outlook: A Preliminary Mapping and its Implications*[27]" offers an analysis based on a more economic and social perspective. Through the investigation of 350 senior risk professionals' perceptions, it expresses particular concern for a prolonged recession of the global economy, high levels of structural unemployment (especially youth) and another possible global outbreak of COVID-19 or other infectious diseases. The report identifies four key areas of concern: Economic Shifts; Sustainability Setbacks, Societal Anxieties and Technology Dependence.

In the RARE 2030 project, the risk of pandemics was taken into account initially as a wild card whereas it now clearly appears that it must be qualified as a structural risk, as scientists have alerted on the increased possibility of new waves of COVID-19 or of new and different pandemics[28].

Going through the above-mentioned foresight reports, it is interesting to note that risks such as increased inequality and reduced cohesion were considered by the RARE2030 foresight study as structural trends – that the health crisis has now markedly deepened. Similarly, opportunities accelerated by the COVID-19 as digital health, AI and multi-stakeholder collaboration in research were already included in RARE 2030 trends analysis. The added value of foresight as a strategic approach to support policy formulation thus appears forcefully confirmed.

[27] World Economic Forum in partner with Marsh & McLennan and Zurich Insurance Group. COVID-19 Risks Outlook: A Preliminary Mapping and its Implications. May 2020.

[28] WHO. The best time to prevent the next pandemic is now: countries join voices for better emergency preparedness.1 October 2020. https://www.who.int/news/item/01-10-2020-the-best-time-to-prevent-the-next-pandemic-is-now-countries-join-voices-for-better-emergency-preparedness

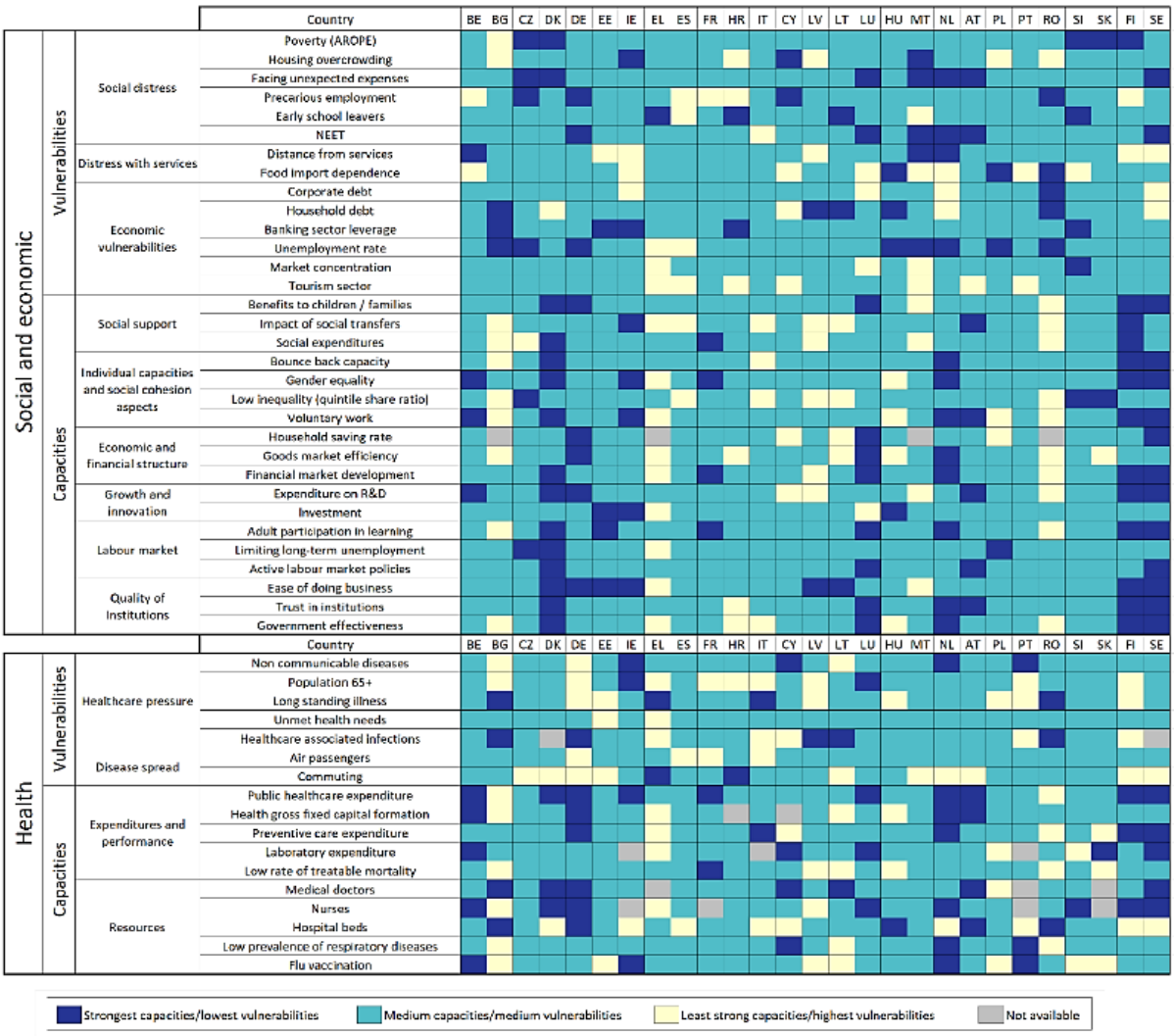



*Figure 9 Prototype dashboard for social and economic resilience linked to the COVID-19 crisis. Source: EC 2020 Strategic foresight report. Charting the course towards a more resilient Europe.2020*

## 2.4 Emerging opportunities for creating resilient health systems

While fighting a pandemic, Europe is on the edge of a new economic, social and political crisis. The ways in which the EU, and each Member State, will react to the crisis will influence how the next decades play out. *To what extent will countries stand together and agree on plans, programs and strategies to ensure not only an economic recovery but a renaissance built upon the principles of social cohesion and solidarity?*

In this regard, lessons can be drawn from the 2008 global financial crisis. Sir Michael Marmot recently wondered "*whether we learned anything from the bad policy choices made after the 2008 Global financial crisis; or, as we emerge from the current crisis, will we repeat the same mistakes with disastrous consequences for health and health inequalities*"[29]. Between 2005 and 2009, EU Member States made huge

[29] Michael Marmot. Twitter Declaration. 9 april 2020.

progress in improving access to health care. The number of people reporting unmet needs fell steadily from 24 million in 2005 to 15 million in 2009. Following the 2008 economic crisis, most Member States have reduced their health care budget implementing sectoral and often-unfair measures whereas few States have taken the chance to start more complex actions that could bring better quality healthcare, increase the access to care and efficiently generate cost savings. By 2013, as result, the number of people reporting unmet needs for health care, especially due to financial barriers, had bounced back to 18 million (3.6% of the population)[30]. The situation disproportionately affected people of lower socio-economic status, those with low health literacy, poor education and generally those with greater healthcare needs[31]. In addition, following the 2008 economic crisis, new vulnerable groups emerged due to the raised unemployment, especially among young men, and due to the increased household debt problems, particularly among young couples[32].

The *EC Joint Opinion Improving pandemic preparedness and management report*[33] underlines as countering inequalities in institutional and legal structures is a crucial part of preparedness and response strategies. *"Policies and practices of pandemic management – if viewed through a lens of equitability – would therefore be focussed on understanding, anticipating, monitoring and minimising the impact of the crisis especially on those highly vulnerable groups"*[34]. The report highlights that "*crisis resilience and preparedness root in societal institutions of solidarity and sustainable long term planning towards stronger equity*[35]".

In line with this, the Expert Panel on effective ways of investing in Health (EXPH) in the opinion "*Organisation of resilient health and social care following the Covid-19 pandemic*[36]*"* identifies the building blocks to create resilient health and social care systems. The document stresses the urgency of paying attention to, and ensuring healthcare provision for vulnerable patient groups. By recognizing the role of primary care and social determinants of health, the opinion underlines the COVID-19 indirect and unintended consequences on vulnerable groups echoing the concept of *sindemia* proposed by Horton[37]. It also stresses the COVID-19 impact in health delivery and organization. With regard to Rare Diseases, the Opinion reports the results of the EURORDIS Rare Barometer survey on COVID-19 impact: Rare diseases patients suffered the disruption of care in many different ways. The crisis brought delayed/interruption of routine treatments administration, appointments for screening tests and certain medical and surgical interventions. The imposed travel restrictions and the hospitals (in the home country or abroad) cancellation of non-COVID-19-related interventions imposed a heavy toll to PLWRD.

To address these challenges the Expert Panel on effective ways of investing in Health recommends to:

- Invest in training and resilience of local health workforce,
- Invest in Research and development,
- Monitoring disinformation,

[30] European Commission.Access to health services –summary of preliminary opinion. 2015.
[31] European Commission.Access to health services –summary of preliminary opinion. 2015.
[32] EUROFUND "Access to healthcare in times of crisis" 2014
[33] EC. Joint Opinion. Improving pandemic preparedness and management. November 2020.
[34] Few et al., 2020
[35] EC. Joint Opinion. Improving pandemic preparedness and management. November 2020.
[36] EC. Opinion of The Expert Panel On Effective Ways of Investing In Health in the Organisation of resilient health and social care following the covid-19 pandemic. November 2020.
[37] R. Horton. Offline: COVID-19 is not a pandemic. The Lancet. Volume 396, Issue 10255, P874, September 26, 2020

- Foster inter-sectoral and inter-system collaboration for health (i.e through the linkability of databases across systems and sectors, in conformity with the GDPR, and with access for patient and providers),
- Reinforce the primary care and mental health systems,
- Reduce social and ethnic disparities in health,
- Provide Specific (inter-professional) training courses that aim at addressing specifically socially deprived and minority health needs.

To enhance the resilience of health systems, the Panel welcomes the European Health Union strategy, announced by Ursula von der Leyen, in her first speech on the State of the Union (16 September 2020). In the same speech, she announced that, under the Italian Presidency of the G20, the Commission would organise a Global Health Summit in Italy to show that Europe is there to protect its citizens.

In a May 2020 opinion piece[38], several MEPs called on the Parliament to set up a body dedicated to solidarity and major public health challenges. Stakeholders from academia, civil society, business community and institutions have elaborated a statement asking to recognize public health as shared competence and to give the EU ability to act on a federal basis in health emergencies.

On 11th November 2020[39], the President of the EC underlined that the European Commission was taking the first steps towards the building the European Health Union – that is intended to strengthen the EU's health security framework, and to reinforce the crisis preparedness and response role of key EU agencies. The two main pillars of the European Health Union are:

1) A stronger health security framework, which will entail:
   - Harmonising European, national and regional preparedness and response plans.
   - An EU emergency system to trigger increased coordination and rapid action to develop, stockpile, and procure the equipment needed to face the crisis[40].
2) More robust EU agencies in terms of competences, budget and staff with the European Centre for Disease Prevention and Control in monitoring the epidemiological situation, the European Medicines Agency in covering the safety of medicines and medical devices, risk of shortages and clinical trials of medicines, and the establishment of a new Health Emergency Response Authority (HERA).

The European Health Union touches on competences at all government levels. According to regional and local actors, achieving it requires the 'active subsidiarity' approach proposed by the European Committee of the Regions (CoR); that is, an intense dialogue across levels of government on the scope of future EU action in this field.

The announcement of the European Health Union has prompted a huge mobilization of European citizens, academia, healthcare workforce, patients' associations, policy makers advocating, as European citizens, to make the most of this great opportunity. In November 2020, a Manifesto for European Health Union[41] has been published calling EU political leaders "*to commit creating a European Health Union*" and to promote a better and major role of EU in health. The Manifesto exposes principles and goals and proposes concrete policies and measures. Inspiring Principles are:

[38] https://www.theparliamentmagazine.eu/news/article/european-parliament-must-listen-to-citizens-applause
[39] EC. Building a European Health Union: Stronger crisis preparedness and response for Europe. 11 November 2020. https://ec.europa.eu/commission/presscorner/detail/en/ip_20_2041
[40] EC. Building a European Health Union: Stronger crisis preparedness and response for Europe. 11 November 2020. https://ec.europa.eu/commission/presscorner/detail/en/ip_20_2041
[41] https://europeanhealthunion.eu/

1. Priority to measures that deliver wellbeing and longer and healthier lives for all Europeans;
2. Precaution, proportionality, and dignity, while also respecting fundamental rights, including equality on any grounds, including sex, race, colour, ethnic or social origin, genetic features, language, religion or belief, political or any other opinion, membership of a national minority, property, birth, disability, age or sexual orientation of gender, ethnicity, or sexuality;
3. Respect for regional and national differences, both in the design and prioritisation of policies, taking account of differing contexts, and in their implementation, taking account of the principle of subsidiarity;
4. Solidarity within and among Member States and with the rest of the world, with measures to safeguard their ability to deliver safe and effective health services. No one is safe until all are safe.

In the document, promoters advocate to develop a European Health Union that strives for the health and wellbeing of all Europeans, focusing on strengthening solidarity within and among MS and providing health and social security for all citizens, especially the most disadvantaged groups. The Manifesto recognises the importance to build strategies and actions on the concept of One Health and environmental sustainability - the latter put forward by European Green. Moreover, promoters ask for giving a major role to citizens "*so that policies that affect their health are created with them and not for them*". The Manifesto encourages a revision of the Health Threats regulatory framework and a major role of EU in health research with the creation of a European equivalent of the US Biomedical Advanced Research and Development Authority (BARDA), a strengthened EMA, and other measures promoting research collaboration across Europe.

Further, the promoters identify a number of measures to achieve the health related Sustainable Development Goals among which: counteracting the unequal distribution of health workforce capacities in Europe, supporting PLWRD and developing a Global Health Policy, working with the UN and its specialised agencies - especially a strengthened World Health Organization - and other multinational organisations contributing to health.

Priorities and policies advocated in the Manifesto are in line with the potential initiatives proposed by European Parliament[42] to strengthen healthcare system across Europe and foster a comprehensive EU public health policy over the medium to long term (Annex 2)

## 2.5 COVID-19 and the Rare 2030 Scenarios

UN Secretary General A. Guterres underlined that coronavirus "*does not discriminate, but its impacts do - exposing deep weaknesses in the delivery of public services and structural inequalities that impede access to them*"[43]. The impact of COVID-19 on Rare Disease Patients is well documented in the RARE 2030 Knowledge Base Summary on Rare Diseases and Coronavirus[44]. As mentioned in the previous section, PLWRD are, in many cases, more vulnerable in case of infection and face increased difficulties in access to care. A full picture of the hardship can be deduced by the EURORDIS COVID-19 Rare Barometer survey[45] answered by 8.551 patients across Europe. The survey highlights that 84% of European rare disease patients surveyed experienced some sort of disruption of their care due to the COVID-19 crisis and around 3 out of 10 respondents reported that this would probably or definitely be life-threatening. Among those who reported a disruption of care: 6 out of 10 were unable to access diagnostic tests; 6 out 10 were unable to receive

[42] European Parliament. BRIEFING EPRS Ideas Papers. Thinking about future EU policy.2020
[43] UN. We are all in this Together: Human Rights and COVID-19 Response and Recovery. 23 april 2020.
[44] Rare 2030 Knowledge Base Summary on Rare Diseases and Coronavirus (to be published)
[45] https://www.eurordis.org/covid19resources

therapies such as chemotherapies or infusions; 6 out of 10 saw their surgery or transplant postponed or cancelled with considerable impacts on symptoms control and quality of life.

Patient organizations have also raised the attention around the risk of discrimination against rare disease patients, and those with disabilities, during the pandemic (i.e in the triage of intensive care), calling to be recognized as priority population for preventative measures. In addition, coronavirus has catalyzed interests and efforts in research: clinicians have less time to conduct research and clinical trials have been hampered.

To face the uncertainty of how to deal with COVID-19, ERNs, national rare disease expert networks and patient organizations have mobilized their efforts to produce information and guidelines, an example of which is the EURORDIS COVID Information Resource Centre[46]. In line with this, the EU Project Share4Rare[47] has launched a new registry for rare disease patients affected with coronavirus (Covid-19) or SARS.

Beyond the emergency, there is a threat of directing public health policy priorities only on a strictly focused COVID-19 response. Conversely, the crisis could lead to build a '*resilience and a post-pandemic recovery rooted on solidarity and collective responsibility*' as recommended by Expert Panel on effective ways of investing in Health (EXPH) and the Manifesto for a European Health Union - mentioned in the previous sections. The instances put forward by these two key documents are in line with the vision underlying the RARE 2030 "Investment for social Justice" scenario. The "Investment for social Justice" vision echoes the calls to build a more inclusive and fair society, promote the coordination role of EU in health policies, foster international cooperation in research. The on-going discussion related to ways to support cross-country and multi-stakeholder collaboration in research, innovation and health provision - even through ERN - builds up on experiences of the rare disease community. In line with current strategies, the RARE 2030 Scenarios recognised the key potential of digital technologies – and AI – to speed-up research, ensure health services and facilitate cross-border healthcare.

As the RARE 2030 Knowledge Summary on Rare diseases and coronavirus underlines "*The current health crisis has brought to the forefront the need to urgently address those pre-existing health, social and economic inequalities that the rare disease community has been tackling for a long time. This has added momentum to the cause of Universal Health Care*". To support this, Rare Diseases International launched a "*Statement on COVID-19 response and recovery*[48]" urging policy makers to "build back better", committing to promote solidarity and ensure Universal Health care when designing Coronavirus policy responses and recovery measures.

[46] https://www.eurordis.org/covid19resources
[47] https://www.share4rare.org/
[48] https://www.rarediseasesinternational.org/wp-content/uploads/2020/07/RDI-STATEMENT-Not-leaving-behind-RDs-in-COVID-19_Final.pdf

# 3. Signs of change: the Pharmaceutical strategy for Europe

The EC adopted the Pharmaceutical strategy for Europe[49] on 25th November 2020 with the aim to represent a "*patient-centred strategy that aims to ensure the quality and safety of medicines, while boosting the sector's global competitiveness*". The strategy intends to analyse the root causes of still present unmet medical needs in medicines availability and accessibility and support the Research and Development (R&D) with greater 'conditionality' of incentives. It is built on the following 4 pillars[50]:

- ensuring access to affordable medicines for patients, and addressing unmet medical needs (in the areas of antimicrobial resistance and rare diseases, for example);
- supporting competitiveness, innovation and sustainability of the EU's pharmaceutical industry and the development of high quality, safe, effective and greener medicines;
- enhancing crisis preparedness and response mechanisms, diversified and secure supply chains, address medicines shortages;
- ensuring a strong EU voice in the world, by promoting a high level of quality, efficacy and safety standard.

The strategy recognised the importance of creating a health data space and ensuring regulatory conditions enabling innovative trial designs. The Communication includes a set legislative and non-legislative action actions, among which:

- revise the legislation on medicines for children and rare diseases to improve the therapeutic landscape and address unmet needs (e.g. in paediatric cancer) through more tailored incentives by 2022;
- accelerate the drug development by incorporating the European Medicines Agency (EMA)'s priority medicines scheme in the regulatory framework in 2022;
- enabling of parallel clinical trial advice from both the EMA and national HTA bodies in 2021;
- facilitate the collaboration of existing committees/networks of regulators, health technology assessment (HTA) bodies and payers to improve the availability and affordability of medicines through the adoption of a Regulation on health technology assessment by 2021.

In 2020, the publication of a comprehensive evaluation[51] of the legislation on rare diseases and children represented a first steps toward the revision. The evaluation recognises that both regulations[52] have "*fostered the development and availability of medicines for patients with rare diseases and for children*[53]*".* However, regulations "*have not adequately managed to support development in areas where the need for*

[49] https://ec.europa.eu/health/human-use/strategy_en
[50] Ibidem
[51] https://ec.europa.eu/health/human-use/paediatric-medicines/evaluation_en
[52] The decision of joint evaluation is due to the fact that even if up to 75% of rare diseases are paediatrics, children suffer the most from the lack of R&D. Among the recognised challenges of R&D for paediatric use are the heterogeneity of the population, hormone status, pharmacokinetics and issues in the ethics of research.
[53] EC. Joint evaluation of Regulation (EC) No 1901/2006 of the European Parliament and of the Council of 12 December 2006 on medicinal products for paediatric use and Regulation (EC) No 141/2000 of the European Parliament and of the Council of 16 December 1999 on orphan medicinal products. August 2020. https://ec.europa.eu/health/sites/health/files/files/paediatrics/docs/orphan-regulation_eval_swd_2020-164_exec-sum_en.pdf

*medicines is greatest. Products tend to be developed in certain more profitable therapeutic areas for which the number of available treatments is increasing*[54]".

The evaluation underlines as both Regulations have successfully redirected private and public investment towards previously neglected areas and catalysed EU and national research programmes in the field of rare diseases. Nevertheless, the document points out that "*not all orphan products authorised under the Regulation are the direct results of such incentives*". It is noted that "*of the 131 orphan medicines authorised in the EU since 2000, the Orphan Regulation is estimated to be responsible for at least 8-24 new ones. The remaining 107-113 products were made available more quickly, and reached more people across the EU, than before the Regulation took effect*". In addition, some products were developed by consortia that could not benefit from incentives due to the fact that *"charitable foundations and academic institutions are not eligible for fee reduction because of difficulties in meeting the 'SME criteria*".

The evaluation stresses that the set incentives were not "*sufficiently effective to catalyse the clinical development to areas where there are no treatments yet. At the same time (..) products tend to be developed in certain more profitable therapeutic areas for which the number of available treatments is increasing*" such as oncology. Here, the market has started to look more similar to 'standard' medicines making questionable whether incentives such as the 10-year market exclusivity are justified.

Rais Lais and Tubeuf conducted a research study on unmet needs using research data from Orphanet and academic publication. The study adopted the following five proxies to identify unmet needs: (a) Nn of research projects; (b) Nn of academic publications; (c) Nn of clinical trials; (d) Nn of orphan designations; (e) Nn of orphan drugs with marketing authorisation across age classes of the disease symptoms. From the analysis emerges an inequality in R&D in high prevalence and high incidence rare diseases. R&D significantly underserved those rare disease diseases that occurs in childhood, those with immediate danger of death or with a high level of uncertainty on the clinical presentation and progression.

On the other hand, the evaluation report also stresses that marketing authorisation at EU level has not always ensured greater accessibility of the authorised medicines for patients in all Member States. Access to orphan medicines still varies considerably and it is highly dependent on factors beyond regulation field such as: different national pricing and reimbursement systems, companies' strategic decisions on market launch, and the role of healthcare providers. In line with this are the findings of the systematic review on RD policies and Orphan Drug Reimbursement Systems conducted in 12 Eurasian Countries[55] by Czech et al.[56].The review registered a wide range of inequality in accessing to new OMPs among RD patients and this is due to differences in national policies, healthcare budgets, health insurance, and reimbursement systems. As example, in some countries - like Netherlands, Germany, and France - nearly all OMPs are reimbursed, in other - like Armenia - none. The review also alerts on trend of imposing stricter rules for the reimbursement of expensive orphan drugs. Similar results were reached in the study conducted by Zamora et al.[57] focused on comparing access to orphan medicinal products in four EU countries[58].

The concentration of R&D in high prevalence and high incidence diseases may partially arise from the knowledge of the *etiology*, the natural history of the specific disease and the small population of ultra-RD available for clinical trials. However, it is the role of policies to support R&D in the most neglected fields and counteracting /balancing companies strategic decisions linked to expected industry returns.

[54] Ibidem

[55] Armenia, France, Germany, Kazakhstan, Latvia, The Netherlands, Poland, Romania, Russia, Turkey, Ukraine, and the United Kingdom

[56] Czech M, Baran-Kooiker A, Atikeler K, et al. A Review of Rare Disease Policies and Orphan Drug Reimbursement Systems in 12 Eurasian Countries. Front Public Health. 2020;7:416. Published 2020 Jan 28. doi:10.3389/fpubh.2019.00416

[57] Zamora, B., Maignen, F., O'Neill, P. et al. Comparing access to orphan medicinal products in Europe. Orphanet J Rare Dis 14, 95 (2019). https://doi.org/10.1186/s13023-019-1078-5

[58] UK, France, Italy, Germany and Spain.

## 3.1 The Pharmaceutical strategy for Europe and RARE2030 Scenarios

The brief analysis provided above underlines as the progresses made by R&D do not currently translate into actual health benefits for the large majority of people living with rare diseases due to issues concerning availability, accessibility and affordability of treatments[59]. This is in line with the recognition from stakeholders of the RARE2030 "Fast over Fair" Scenario as the baseline one. Without any social, economic, political changes (at European, national and regional level), this is the future we will live in.

Under this Scenario, described in D5.2, rare diseases are researched and OMPs rewarded as long as their orphan status is recognized as profitable. The R&D is concentrated in certain rare diseases areas, while very rare and complex diseases are left behind. Even if incentives are set to support the successful market launch of products and shorten the cycle of R&D, innovation achieves maximum results with minimum efforts. There is a focus on efficiency rather than on piloting breakthroughs. Developers impose high prices and reimbursement process remain highly different among EU countries. In addition, in this Scenario, the take-off of cross-country infrastructure to collect and share data to foster research, as the European Platform on Rare Disease, has proven harder than expected and it is partial and fragmented.

This Scenario can be enriched by the analysis carried out by the Imperial College of London in the framework of RARE2030 (D5.3)[60]. The comprehensive study first maps historically the differences in innovation for orphan drugs versus non-orphan drugs, by disease area, ATC, region, and over time. Secondly based on these the observed heterogeneity of innovation across disease, it measures inequalities in product innovation targeting rare diseases. Finally, based on these historical trends, the quantitative analysis provides a forecast of the stock of innovation to be made available over the next ten years.

While the detailed information on methods, data and results are included in D5.3, we will include in the next paragraphs findings extracted from report main messages that could serve to inform the "Fast over Fair" Scenario[61].

- *In a retrospective analysis, the study examined the stock of innovation, both orphan and non-orphan, made available between 1980 and 2019. By looking at the distribution of unique products by disease area for orphan products, across early stage R&D, late stage R&D and market launch, it is revealed that more than half of orphan products (53%) targeted cancer, followed by endocrine and metabolic disorders (12%) and cardiovascular disease (9%)[62].*
- *Similar findings were found in the retrospective analysis related to the percentage of products across all stages by ATC separately for orphan products. The results reveal that more than half of all orphan products being antineoplastic and immunomodulating agents (53%; ATC class L), followed by products targeting the alimentary tract and metabolism (11%; ATC class A) and the blood and blood forming organs (9%; ATC class B).*
- *Based on the historical data and in the absence of any policy changes, the report also provides a forecast of the launch of between 675 – 807 orphan designated products between 2020 and 2030. Most new orphan designated products will target cancer, cardiovascular disease, endocrine and metabolic disorders and musculoskeletal disorders, while almost no new orphan designated products expected to target ophthalmological, genitourinary, dermatological, gastrointestinal, respiratory and CNS disorders.*
- *The analysis also compares inequalities in R&D activity and market launches across parent categories and measures the level and direction of inequalities for each patent disease separately.*

[59] RARE2030, EURORDIS Policy Recommendation "Available, Affordable and Available Treatments" DD6.1

[60] RARE2030 ICL D5.3"Report on the results of the quantitative analysis of R&D and market launch of orphan drugs"

[61] Ibidem

[62] Ibidem

*Both analyses consider data from 1980- 2019 and two distinct periods – pre-2000 and post-2000 - reflecting the period prior and post European Orphan Medicinal Product Regulation. ICL study shows that across all rare disease there is inequality pro-occurrence for early- and late-stage R&D and market launches with activity disproportionately concentrated towards the more prevalent diseases. Looking at each group of diseases though there is heterogeneity in the levels of inequality. Market launches are distributed in proportion to need for several rare disease groups over the entire period[63]. However, for other disease areas most recent data shows that there is pro-occurrence inequality (i.e., disproportionate level of market launches concentrated towards high prevalence disease)[64]. These disease areas also exhibit pro-occurrence inequality for early and late stage R&D. For most of these disease groups inequality has remained pro-high prevalence but has reduced after the implementation of the European Orphan Medicinal Product Regulation.*

- *The study analyzes also the distribution of clinical trials conducted between 1996 and 2016 for drug treatments of rare and non-rare cancers. The quantitative analysis shows a higher level of inequality for rare than for non-rare cancer trials that is unexplained by the prevalence and/or the incidence of the cancer. It emerges that almost 60% of rare cancer types have no corresponding clinical trials in the study period, as trials are concentrated on high prevalence and high incidence rare and non-rare cancers.*
- *Inequality has increased substantially between 2000 and 2016 within rare cancers, while staying at about the same level within non-rare cancers over the same period. This result shows that, even though the number of rare cancer clinical trials has increased since 2000, not all rare cancer types benefited equally from this trend. A few cancers, with higher incidence and prevalence tend to disproportionately benefit of more rare cancer research, the 20 most funded rare types making up for as much as 75% of all rare cancer observations (RD) and 45% of all rare cancer trials (RWD). Unfortunately, these 20 cancer types only account for around 29% of the prevalence and incidence of rare cancers, leaving the other 71% of cumulative prevalence and/ or incidence with only 25% of rare cancer research.*

Against this baseline Scenario, stand the RARE2030 Vision "Innovation for Social Justice" – the most desired scenario by the stakeholders consulted during the foresight process. Under this Scenario, multi-stakeholder initiatives prioritize RD investments in innovation that meet PLWRD' needs, focusing on as many diseases as possible and not only on low hanging fruits. Here, research is increasingly *mission-oriented*[65] and governments at all territorial level (Global, EU, national) are coordinated in setting incentives to goad and reward effective innovation. Under the EU guide, the set incentives are targeted toward the most needed/neglected areas and the process of accessing incentives is open to conventional actors ("beyond the SME criteria"). Multi-stakeholder partnerships are encouraged: citizens and patients are considered equal partners in research with scientists and healthcare professionals, public funders, private enterprises. Patients' early involvement in setting priorities and design research improves patients trust, recruitment and retention in research and development projects. Patient-relevant outcomes and experiences are systematically evaluated and are essential part of research. Thanks to international collaboration, advances made in next-generation sequencing (NGS) technology that allows approaching rare and ultra-rare genetic disorders with a more accurate diagnosis. The development of OMPs reaches and goes beyond the goals set by IRDIRC increasing the availability of products for diseases without current approved options. Existing medicines and treatments are affordable and equally available no matter where PLWRD live in Europe.

63 namely: bone disease, cardiac disease, circulatory system disease, developmental defects during embryogenesis, endocrine disease, renal disease, respiratory disease, and skin disease.

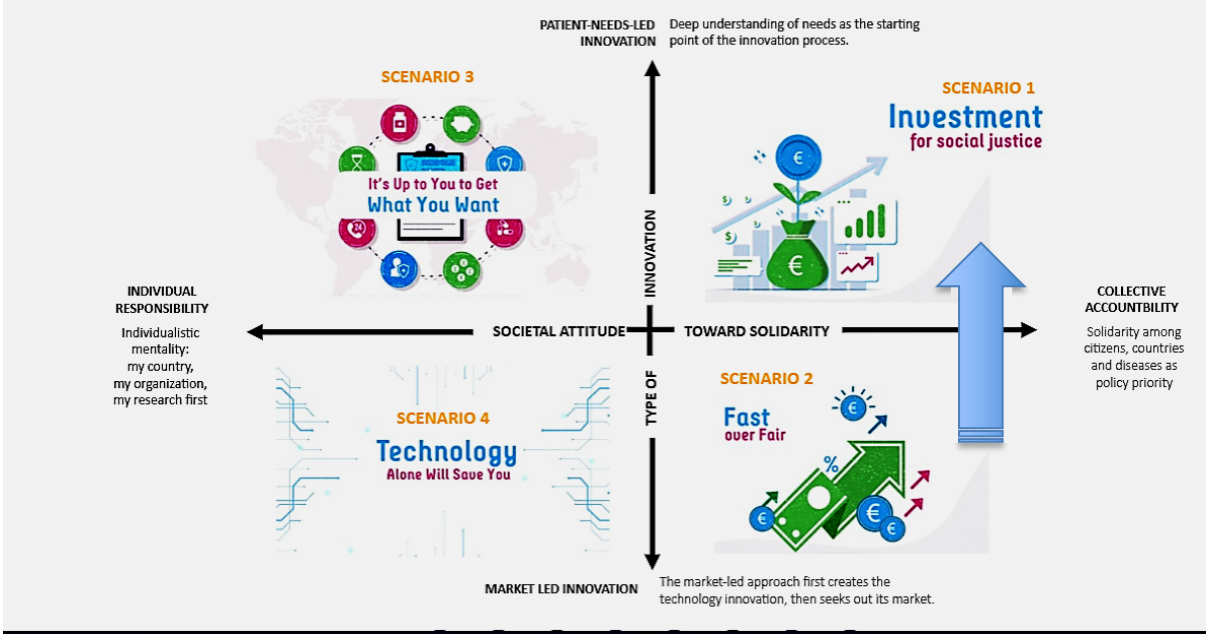
64 Namely systemic or rheumatologic disease, inborn errors of metabolism, hepatic disease, hematologic, neurologic disease, and ophthalmic disorders

65 M. Mazzucato et al. UCL. The people's prescription. Re-imagining health innovation to deliver public value. UCL, 2018

The RARE2030 Recommendation "Available, Accessible and Affordable Treatments"[66] set the following goals to be achieved under "Investment for social Justice Scenario" by 2030

- *More and better quality curative, stabilising, palliative, assistive, rehabilitative and preventative technologies and therapies are available, accessible AND affordable for all people living with rare diseases in Europe*
- *Europe is a world leader in the development of rare disease therapies with a competitive regulatory ecosystem and a more robust pharma and biotech manufacturing presence, leading to greater investments in research and product development, with accompanying improvements in patient access and health monitoring*
- *1000 new therapies should be available by 2030, in line with the IRDiRC vision*
    - *Treatments should be approved in the EU for 500 different rare diseases and for 50% of the overall population of people living with a rare disease.*
    - *These new treatments and technologies should focus on unmet needs with two goals:*
        - *Curative, transformative or stabilising and symptomatic treatments for 200 of the 400 most frequent rare diseases covering over 90% of the population living with a rare disease;*
        - *Curative or transformative treatments for at least 100 rare diseases from the group affecting less than one in 100,000*
- *Therapies should be 3 to 5 times more affordable than currently available treatments*

The document also contains a clear definition of the pathway – the steps needed to be taken by the different actors to move from the most probable scenario (Fast over Fair) toward the most desired one "Investment for Social Justice".




[66] RARE2030 EURORDIS "Available, Accessible and Affordable Treatments"

The RARE2030 recommendation[67] highlight that multi-sectoral and coordinated policies and actions are needed to be adopted and implemented at different territorial levels and by all stakeholders involved to reach "Investments for Social Justice" scenario and ultimately improve the health outcomes and quality of life of people living with rare diseases.

The recommendation – built on a wide consultation of stakeholders – recognizes the need to:

*Establish streamlined regulatory, pricing and reimbursement policies. These policies should encourage a continuum of evidence generation along the full life cycle of a product or technology as well as the patient journey from diagnosis to treatment access. A European ecosystem able to attract investment in areas of unmet need, foster innovation, and address the challenges of healthcare system sustainability.*

*Specifically, policies should enforce:*

- *early-stage multistakeholder identification of unmet needs and subsequent priorities and investments*
- *a threshold of eligibility: including prevalence of no more than 5/10,000 individuals (an incidence for rare cancers of less than 6 per 100 000 per year) and avoids artificial breakdown of common diseases into rare subsets*
- *a graduated system of incentives, rewarding earliest dialogue and favouring areas with no therapeutic options (currently disregarded diseases)*
- *a strengthened mandate for the European Medicines Agency Committee for Orphan Medicinal Products encompassing early dialogue, designation, ongoing scientific advice and protocol assistance, scientific qualifications (in particular of registries and patient-centered outcome measures (PCOM)), risk-benefit assessment, and post-marketing requirements*
- *a functional and efficient EU HTA Framework to support the assessment of effectiveness and relative effectiveness (and, in the interim, incentivisation of joint EMA/HTA assessment at the European level and uptake at the national level)*
- *a continuum of comparative evidence generation throughout the product life cycle and patient journey, enabled via multi-purpose disease registries and all other relevant data sources*
- *a European Table of Pricing and Negotiation enabling European collaboration between Member States*
- *an EU-Fund to co-finance the generation of evidence across EU Member States and reduce uncertainties during the first years following approval, for advanced therapies for the rarest diseases (affecting less than 1/100 000.)*

[67] RARE2030 EURORDIS "Available, Affordable and Available Treatments"

# ANNEX I - RARE 2030 SCENARIOS

The table below provides a snapshot of how the trends identified in step 2 of the RARE 2030 Foresight study could evolve under the four different Scenarios whereas the following four sections describe in greater detail the four different possible futures shaped in the scenario building phase.

| | 1 Investments for social justice | 2 Fast over Fair | 3 It's up to you to get what you need | 4 Technology alone will save you |
|---|---|---|---|---|
| SOCIAL ATTITUDE | EU increased cooperation on health policies | EU increased cooperation on health policies | Health is a national issue, less cooperation and solidarity between countries | Health is a national issue, less cooperation and solidarity between countries, citizens and patient organisations |
| INNOVATION | Population needs led innovation | Technology led innovation | Population needs led innovation | Technology led innovation |
| Rise of multi-stakeholders governance | ERNs are thriving – they are the centerpiece of RD and specialised healthcare scene in Europe | ERNs active but more as European administrative –structures, used more by pharmaceutical companies than by patients | Strongest power for ERNs of diseases/groups of diseases with more political engaged patients and/or industries connections | ERNs no longer exist - Cooperation collapses under financial strain |
| Rise of innovation-oriented research | Multi stakeholders, cross countries research led by government | Multi-stakeholders, cross-countries research led by industries | Powerful groups and empowered individuals lead research | Few powerful private companies lead the research |
| Medical Innovation and genomics | Technologies cover unmet needs, including also the needs of those diseases that are typically underserved | Innovation try to achieve maximum results with minimum efforts. Companies and regulators strike a balance between what is needed and what is profitable with redundancy of treatments for the most known diseases. | Focus on local needs and on those diseases of the most empowered groups. | No coordination between stakeholders, the market decides focusing on profit rather than needs. |
| Healthcare systems and new care delivery models | Needs-led, outcomes-driven system, Specialised and primary care fully integrated ensuring continuity of care. Holistic care is the pivotal principle of healthcare | Increased cost-saving policies led to top-up payments for breakthrough innovations. High quality specialized services (i.e RD Centre of expertise) exist but there is lack of knowledge in primary care. | Wide differences on healthcare services provision and quality among EU regions. Holistic care pathways often established thanks to patient organisations. | Private, insurance based healthcare systems. Healthcare professionals have no time to investigate the complexity of PLWRD. Centers of Expertise reduce paramedical and holistic care services. |

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| Digitalisation of healthcare | Digital revolution of the healthcare system accomplished with equal role of all stakeholders (government, patients, private companies) | NHS rely on private companies for digital skills. National infrastructures are often too old for the new digital innovation that not always meet patients' needs | Companies and patients are allies to develop and improve digital solutions useful to patients | Technologies depersonalise healthcare without real outcomes improvement |
| Standard and interoperable data and the rise of AI | EU sets rules and builds common infrastructures for data sharing and the implementation of AI in MS | Soft government ensure some common rules to regulate the market which offers services and infrastructures | Bottom-up approaches – empowered communities set rules and infrastructures to best meet their needs | Few private global companies manage the structures and infrastructures |
| Access to treatment and care | Harmonisation of HTA at EU level and cooperation and transparency in pricing and reimbursement lead to equal access to treatment across Europe | Cooperation and transparency is a principle that does not translate in effective policies. The cost of OMPs and RD devices remain a barrier for many to access treatments and care. Innovative drugs take long time to reach patients | Treatment and care availability depends on country/ individuals/groups willingness to pay or reach agreements | Treatment and care availability depends on individuals willingness to pay or charity foundation support |
| Equity and solidarity | "Leaving no one behind" is a must for health policies at all territorial levels | EU collaboration focus on technological development – little attention payed to social inclusion, psychological and educational measures | Increased solidarity only for those of the same community/coalition (disease, territorial, category) | Increased competition between citizens and groups. Worsening of equity, exclusion in society, discrimination in the labour market for vulnerable population |
| Ageing of population | Harmonisation of NBS among EU countries and services set to guide the age transition | Establishment of rules for NBS market and exchange of best practices for guiding age transition | Wide difference across countries and regions on the availability of NBS and services offered for elderly | Only the better off across EU countries access to NBS and specialised services for the elderly. that are often out of pocket. |
| Advocacy evolution | Trained and empowered patients collaborate in a systematic way with multi-stakeholders team | Trained, empowered patients work with and received different forms of incentives (money, share) | High professionalism of the most empowered patients groups, increased competition and divisions among RD community | More trust on technologies than on human groups/knowledge. |

# ANNEX 2 POTENTIAL INITIATIVES FOR FUTURE EU PUBLIC HEALTH[68]

| | Initiative | Likely lead EU actor | What could be done? | |
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| | | **Response to health threats** | | |
| 1 | Tackling medicine shortages and a pharmaceutical strategy for Europe | EU institutions, Member States | During the coronavirus pandemic, the Commission produced emergency guidelines for the rational supply of medicines on 8 April 2020. Given their non-binding nature, it is not clear to what extent Member States will adhere to these guidelines. The pandemic however, has made clear that better cooperation between the Commission, the EMA, the Member States, and the pharmaceutical industry is key in tackling medicine shortages. The EU4Health programme provides support in this direction. Hopefully, the lessons learned from the pandemic with regard to crisis-preparedness and functioning supply chains will be taken on board in the proposed pharmaceutical strategy for Europe, for which the Commission published its roadmap on 2 June 2020. | |
| 2 | Broadening the ECDC mandate and establishing common European standards for health data interoperability | European Commission, Member States | The ECDC's visibility is not matched by legal powers or capabilities to intervene. The ECDC is not a single European centre in the same way as the Centers for Disease Control and Prevention (CDC) are in the United States of America. The ECDC employs less than 300 staff, far fewer than the 9 000 employed by the CDC, and with a limited budget (€60.4 million for the 2020 financial year). In an April 2020 resolution on the coronavirus pandemic, the Parliament calls for the competences, budget and staff of the ECDC (and the EMA) to be strengthened. Also in April, ENVI Chair Pascal Canfin wrote to the Croatian Council Presidency, calling for ECDC staff numbers to be increased.<br>As also suggested in the Franco-German initiative for the European recovery from the coronavirus crisis, the ECDC mandate can be expanded, giving the agency more executive powers to manage technical and human resources against future epidemics, and establishing common European standards for health data interoperability (e.g. harmonising methodologies to make epidemiological statistics more usefully comparable). | |

[68] Source:EPRS Ideas Papers.Thinking about future EU policy.2020

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| 3 | RescEU and the Civil Protection Pool | European Commission | RescEU and the Civil Protection Pool are tools that could be better and more widely used. Member States retain their autonomy and resources, both in principle and in practice. The EU4Health programme would revamp the role and budget of RescEU, and in the face of increasing natural and man-made crises, better coordination, joint work, and pooling of resources are needed. | |
| 4 | Strengthening transnational regional networks during epidemics | Member States, European Commission | The coronavirus pandemic has overloaded the national healthcare systems of many Member States. However, a number of regional initiatives made it possible for Italian and French Covid-19 patients to be treated in Germany and Luxembourg. The Commission provided guidelines, calling on national, regional, and local health authorities to enable health professionals to work across borders and to pool hospital bed capacity. The Commission offered support by coordinating activities through the HSC and the EWRS. Transnational regional networks in the course of epidemics, particularly between neighbouring regions, involving patients and health personnel should be strengthened. | |
| 5 | Extending the joint procurement tool and creation of an EU public procurement agency | Member States, European Commission | The coronavirus pandemic has shown that the joint procurement framework should be strengthened during emergencies and also expanded to use outside them. In 2017, the European Parliament called upon the Commission and the Council to develop new measures, including voluntary joint procurements, to ensure affordable patient access to medicines. Several Member States have engaged in regional collaborations, such as the BeNeLuxA initiative, and the Valletta Declaration. In April 2020, the expert panel on effective ways of investing in health launched a discussion on how to make procurement contribute to better health outcomes. A common EU public procurement agency could provide additional support to ensure more efficient public procurement. In particular, for health expenditure, an EU agency would enjoy greater bargaining power when negotiating with large global suppliers. | |
| 6 | Wider use of structural funds in health | Member States, European Commission | The negotiation of the 2021–27 EU Multiannual Financial Framework is on-going at the time of writing. Given the overall pressure on public budgets, and the emergence of the structural funds as an increasingly important source of capital investment, structural funds should be better and more widely used to make health a priority issue. | |

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| 7 | Extension of the European Reference Networks (ERNs) beyond rare diseases | European Commission | The ERNs, established by Directive 2011/24/EU, are virtual networks involving healthcare providers across Europe. They aim to facilitate discussion between health professionals about complex or rare diseases. In 2018, the Commission expert panel on effective ways of investing in health suggested enlarging the ERNs' mission and widening their scope to other diseases. The EU4Health programme states that the extension of ERNs beyond rare diseases to communicable and non-communicable diseases should be considered. | |
| 8 | From curative medicine to preventive medicine | Member States, European Commission | The potential of public health is limited by multiple factors, including an over-emphasis on curative medicine. The growing personalised medicine agenda and the continuing emphasis on treatment have captured the imagination of politicians and funding agencies. The result has been an on-going diversion of resources towards individualised actions and away from broader population-based approaches. Healthcare today often requires a strengthening of the social welfare system, without resort to sophisticated technological discoveries. Real positive impact on the health of the population can occur through simple measures: more widespread prevention, better basic services, better communication with citizens, and ensuring an appropriate number of health workers. | |
| 9 | Commission Vice-President for Sustainable well-being and a dedicated Commissioner for Health | European Commission | The challenges of the coronavirus crisis provide an opportunity to demonstrate to European citizens that EU institutions regard effective health policy as a high priority. One way of doing this would be give health policy greater visibility and leverage within the European Commission, by designating a Commission Vice-President for Sustainable Well-being and a dedicated Commissioner for Health. | |
| | **Enhancing evaluation of EU health policies** | | | |
| 10 | Foresight and public health | EU institutions | Understanding and being prepared for the future is crucial for public health. In line with the inclusion of a foresight portfolio in the new Commission under Vice-President Šefčovič, EU public health should use foresight tools to improve evidence-based policy-making and better involving the media to raise awareness about future health trends. | |
| 11 | Enhancing impact assessment of public health policies | EU institutions | Regular ex-ante and ex-post evaluation of public health policy strategies proposed by the Commission can be developed and routinely deployed. This could be developed, starting with sectors most related to health, such as research, environment, agriculture, trade, transport, urban planning, and cohesion policy. | |

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| 12 | Better integration of public health issues within the European Semester | EU institutions | According to the Commission's 2020 country-specific recommendations issued under the European Semester, the coronavirus pandemic has exacerbated existing structural challenges related to effectiveness, accessibility and resilience of health systems. Although the European Semester works on fiscal rules, it can better integrate public health issues. In recent years, social and health issues have been progressively included within the European Semester. Current health attention should move to include the capability of public health structures to deal with crises, and incorporate more health and social aspects in general, as these factors will increasingly influence the future financial sustainability of EU countries. | |
| | **The EU as a global health actor** | | | |
| 13 | | | The 2019 Council conclusions on supporting the SDGs across the world note that progress needs to be accelerated, in particular in integrating SDGs more | |

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| | Create government priorities within the framework of the Sustainable Development Goals (SDGs) | European Commission, Member States | closely into existing frameworks. The SDGs make it clear that health and well-being should be part of overall development programmes across all sectors of governance and policy. In practice, the aim is to create government priorities, policies, and budgets that are health-oriented, based on health impact assessments, and focused on sustainability within the framework of the SDGs. The Commission is well positioned to give support, but the outcomes of its actions will require scrutiny. | |
| 14 | Implementation framework for the One-Health approach | EU Institutions | Recently, 11 000 scientists from more than 150 countries issued a document declaring that human consumption and corporate over-reach were degrading ecosystems and driving more than a million species to extinction. This document also stated the importance of the One-Health approach, recognising the interconnection between human health, animal health, and the environment. The EU4Health programme seems well positioned to support Member States in implementing a framework for a One-Health approach. Progress will have to be monitored, since the resources available to the EU4Health are insufficient to meet this objective, and other objectives that the programme proposes. | |
| 15 | Rethinking European engagement with global health | EU institutions | Global health has always been political, and coronavirus has exposed divisions over implementation. As suggested by the Council trio presidency work programme, global health could become a key component of EU policy - not a 'charity programme', but a persistent concern requiring a radical change in multilateral cooperation, particularly with low- and middle-income countries, where access to basic health services remains extremely limited. Without an effective global health approach, factors such as increasing communications, rapid third world urbanisation and inconsistent healthcare systems create conditions for infectious disease outbreaks, making global epidemic surveillance and control systems ineffective. | |
| 16 | Strengthening the role of the World Health Organization (WHO) | EU institutions, Member States | Without better global health governance, specifically strengthening the power of the WHO, even major efforts at EU level will not be enough. Only by acting collectively can the EU and its Member States influence WHO actions. Although the WHO's position has been undermined during the coronavirus pandemic, Member States can still use the authority of the WHO to justify difficult public health decisions, such as implementing the extensive lockdown during the crisis. | |

| Strengthening primary care, e-health and the role of health workers | | | | |
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| 17 | Improving primary care and introducing a 'proximity welfare' model | Member States | Improving primary care requires investment in specialist primary care, the development of new models of shared care, and investment in information and communications technology. In addition primary care should develop a more preventive attitude, as the current focus is on curative care. In the present crisis, it could be useful to implement a 'proximity welfare' model, i.e. physical and virtual meeting and orientation | |

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| | | | locations in metropolitan areas and in towns with 50 000-100 000 inhabitants. The objective would be the multidisciplinary tackling of social problems (early school leaving, dependence, gender violence) through ad hoc interventions where necessary. |
| 18 | Improving role and general conditions of health personnel | Member States, European Commission | Health and social systems within the EU employ more workers now than at any other time in history. Many EU countries report both difficulties in retaining and recruiting health staff. This is becoming increasingly urgent, as healthcare demands grow and the health workforce shrinks. Action needs to be taken at different levels: education (e.g. allocating sufficient time for continuous health professional development); regulation (e.g. reviewing practice of healthcare professionals); finance (providing more incentives and better salaries); and professional and personal support (e.g. introducing specific measures for employees with children). |
| 19 | Digitalisation, the digital single market, and public health | Member States, European Commission | The digitalisation of the healthcare sector forms part of the EU digital single market strategy and measures are already under way. A number of barriers currently hamper the wider uptake of e-health solutions in Europe, including a lack of confidence in e-health among patients and healthcare professionals; limited interoperability; limited evidence of the cost-effectiveness; and a lack of transparency in collecting data. For health systems to evolve equitably and sustainably, it is important to exploit the potential of digital solutions in realistic and inclusive ways. This means being honest about limitations and ensuring that digital solutions address concrete problems faced by patients and health professionals. A danger is that digitalisation could lead to more exclusion, creating larger gaps between socio-economic groups. |
| 20 | Health Technology Assessment (HTA) | Member States, European Commission | HTA assesses the effectiveness of health procedures and technologies. HTA is problematic politically, and also meets opposition from industry and providers. In 2018, the Commission proposed legislation to institutionalise HTA at EU level. The proposal would provide the basis for permanent EU-level cooperation in four areas: convergence in HTA procedures; reduction of duplication of efforts for HTA bodies and industry; ensuring the uptake of joint outputs between Member States; ensuring long-term HTA sustainability within the EU. Member States have objected on several grounds, including subsidiarity. At the time of writing, the legislative process is pending. The coronavirus crisis could act as a catalyst for a real 'Europeanisation' of HTA. |

| | Research and innovation for health | | | |
|---|---|---|---|---|
| 21 | More resources for public health research | Member States, European Commission | Europe is a natural laboratory for health policy and health systems research. With multiple systems to finance and govern healthcare across the 27 Member States, there are many opportunities for international comparative analyses and research. These opportunities can make important contributions to national policy development and bring about consistent improvements for all EU health systems. Horizon Europe should provide more resources for public health and health system research. | |
| 22 | Improving research infrastructures | European Commission | European research infrastructures are facilities that foster innovation and provide resources and services for scientists to conduct research. Key objectives are to reduce fragmentation within the European research eco-system, to avoid duplication, and to combine skills and data in response to global challenges. With the objective of strengthening the ERA, and as stated in the work programme of the Council's trio presidency, the Commission and Member States can work towards improving research infrastructures for the development of new medicines and vaccines. | |
| 23 | Redefining and strengthening training in public health | Member States | Today's public health professionals should engage with all the determinants of health. Public health approaches should be defined centrally, regionally and locally. The identity of the locus and the organisations involved will differ from country to country, depending on the context and circumstances. Public health needs to be an attractive career option: training for public health personnel should be redefined, supporting national public health institutes and universities. | |
| 24 | Disruptive innovation in healthcare | Member States, European Commission | The expert panel on effective ways on investing in health describes disruptive innovation in healthcare as, 'a type of innovation that creates new networks and new organisations based on a new set of values, involving new players, which makes it possible to improve health outcomes and other valuable goals, such as equity and efficiency'. Disruptive innovation changes organisational structures and cultures. Actions aimed at promoting disruptive innovation in healthcare should focus on facilitating factors and removing barriers. The EU could create more protected spaces where healthcare innovators can experiment with new practices, along the lines of innovation deals. | |