



## APPENDIX to the

Advice for 2018–2020 of the Horizon 2020 Advisory Group  
for Societal Challenge 1,  
"Health, Demographic Change and Well-being"

## APPENDIX

### AG reply to stakeholder comments

The Advice for 2018-2020 of the Horizon 2020 Advisory Group (AG) for Societal Challenge 1: “Health, Demographic Change and Well-being” was finished in the beginning of June 2016 after an efficient and productive writing period of 4 months of the expert members of the Advisory Group. The extensive report has 15 themes relevant for future health research.

In mid-July, stakeholder organisations were invited to send their comments of the content of the report. The members of the AG perceived these comments as very important and read these carefully. Next, in September, the AG met with the Programme Committee (PC) members for discussing the report and exchanging their views on the next health research challenges to be tackled by the EU. The members of the AG presented the 15 themes with short summaries of the comments during this question and answer session.

In general the AG advice report has been very well received. The AG wants to thank the stakeholders for their contributions and the PC members and NCPs for their effortful collaboration. Motivation among all involved has been high. The AG appreciates all responses and the volunteer time spent on developing the Report and the Appendix. Thus, the feedback process of the content of the report has been a very constructive and positive one.

Below, short summaries of the written stakeholders’ comments and oral comments from the PC upon the 15 themes as presented September 21st 2016.

#### VERTICAL themes

- 1 Personalized medicine
- 2 Rare diseases
- 3 Infectious diseases
- 4 Non-communicable diseases
- 5 Paediatrics
- 6 Public health and prevention including migration
- 7 Active and healthy aging

#### HORIZONTAL themes

- 8 Big data
- 9 eHealth, mHealth, ICT
- 10 Integration of care
- 11 Environment and health, green solutions and sustainability including climate change

#### CROSS-CUTTING themes

- 12 Social Sciences and Humanities, integration, inequalities, migration and ethics
- 13 Sex and gender differences in medicine

14 Commercialization within “Health, Demographic Change and Well-being”

15 Encouraging stronger and successful involvement in EU-13

Short summaries of written stakeholder feedback and feedback from Programme Committee members in Brussels on 21 September 2016:

## **VERTICAL themes**

### *1 Personalized medicine*

There has been an overall positive reception of the AG report on Personalised Medicine (PM). Programme committee (PC) members reiterated some of the points raised in the PM advice, which they considered particularly important. Specifically, PC members from Norway, Austria and Netherlands emphasized the importance of supporting and coordinating the patient registries and large prospective cohort studies, which are considered a competitive advantage of Europe in the domain of PM. PC members from France and Denmark highlighted the importance of understanding the disease mechanisms, which is crucial for innovation in diagnostics and therapy.

### *2 Rare diseases*

The theme of rare diseases received much interest and support from the stakeholders.

In the written feedback comments, all key elements reported in the Advisory Group’s report were highlighted as important, spanning from the need to support fundamental research on rare diseases, to the elaboration of effective clinical trials meeting the challenge of the small number of patients. Supporting the development of advanced therapies as well as the repurposing of existing drugs for rare diseases were both seen as crucial. It was acknowledged that rare diseases call for coordinated actions engaging all stakeholders involved: patients, payers, regulators, and pharmaceutical and biotechnological enterprises. Overall, the need for a comprehensive pan-European approach supporting coordination and integration of rare disease research.

In the Q&A session with the PC members it was clarified that the science and technology gaps associated with rare diseases also include innovation gaps; the underlying theme of big data generation and handling was mentioned as an example. It was also highlighted that structuring specific calls for rare diseases will be best achieved through coordination and collaboration among funding bodies at European level and beyond, in line with the goals of the International Rare Diseases Research Consortium, IRDiRC.

### *3 Infectious diseases*

The Programme Committee provided a very good feedback on the Infectious Diseases chapter of the AG report; their review and very constructive comments and suggestions were acknowledged. Overall, member states’ representatives agree with AG analysis and recommendations that specially highlight the need to support (i) the development of vaccines against infectious diseases affected by microbial resistance (AMR), (ii) to address the topic of specific and emerging and re-emerging diseases and to (iii) to support innovation in this field namely investing in preclinical, translational and product development research. The PC comments and Q&A session indicate general consensus that point to (a) the imperative need to keep focus on Antimicrobial resistance, (b) the need to develop quick and easy to handle point-of-care diagnostics, looking also at

diagnoses and surveillance, (c) the importance of R&D to be ready for emerging and re-emerging disease threats and (d) the need for improved processes and standards to support innovation - these are all areas highlighted as priorities in the AG report. The importance of supporting projects on a ONE health approach, the articulation with other funding programs in the ID and Vaccines arena, the need to support projects in basic research, namely in disease mechanistic studies of the immune system, in technologies allowing for cost-effective vaccine production and of safe delivery systems were discussed during the Q&A session. Prioritization and the basic research being supported by other EC funding instruments were mentioned. Generally the use of prizes as a funding instrument was well received.

#### *4 Non-communicable diseases*

Following useful feedback from stakeholders, the working group would like to highlight the role of nutrition as an important factor in the growing prevalence of NCDs. With regard to prevention, the group would like to draw a distinction between ordinary prevention (lifestyle intervention) and secondary prevention – to minimise potential discrimination or stigmatisation of patient cohorts. We would like to restate the importance of striking a balance between research into susceptibility and resilience factors for disease manifestation and progression. There needs to be an important emphasis on prevention, screening and early diagnosis, and the development of improved tools for diagnosis beyond laboratory based and including medical imaging as well as therapeutic approaches through minimally invasive interventions and the use of medical devices. In addition, it is crucial to stress the importance of prioritizing studies on NCDs that make use of large cohorts, including but not limited to large bio banks and population registries, to improve quality of treatment and integrated care.

A concern remains that the WG advice offered in the NCD chapter is very broad. The committee worries that without focus, any calls are likely to attract many applicants and possibly be oversubscribed. Some prioritisation of disease categories would be welcomed.

#### *5 Paediatrics*

Prevention is a key issue. Disease mechanism are starting early in life and have impact throughout life. Early disease detection (facilitated through material sampling procedures adopted to very young age groups reducing harm and pain) ,improved intervention and ongoing surveillance ( modern surveillance instruments including health care surveillance passport for children with “special” needs, smart ICT solution including i.e. gaming,) are key to improve quality of life aspects from early on. This has the potential to reduce cost in health care during the course of life. The integration of early life aspects requires also research into pregnancies at risk, pre-terms and neonates. Broadening of themes is necessary. Systems medicine and quality controlled big data have all huge potential to bring major insights and advancements to a broad scope of disease starting early in life and affecting the population lifelong. This includes mental disabilities, epilepsy and autistic children but even more environmental factors triggering pulmonary disease (allergies), diabetes and obesity. Networks connecting enhanced registries outcome data with biobanks under data protection safeguards need to deepen the understanding in particular, but not only, in poor prognosis diseases like cancer. Research into life course models and registries were underlined in comments. To reduce health disparities in Europe visionary eHealth models are needed (HEALTH meets RESEARCH; health surveillance and/ or survivorship passport). European Reference Networks (ERN) is ideal platforms to foster research in rare disease areas. In addition they have the potential to drive population based REGISTRIES to the next level, summarizing key disease data with

outcome data. Interoperability of proposed eHealth solutions is important. NON-pharmaceutical approaches are fundamental areas for young children and people to improve quality of life during life span: Health and social care must have a focus on this vulnerable age groups and foster cognitive developments and improved life styles. Cross cutting research in mode of action (MOA) driven drug development is a key to bring personalised and precision medicine to children and young people. Although the IMI2 call 2016 covers a few aspects, this is by far not enough to suffice the imminent drug needs within the spectrum of diseases in the young population after having been left behind over many years.

#### *6 Public health and prevention including migration*

An integrated understanding of personal, social, and environmental risk factors for health and well-being through the life-span has been emphasized. For a holistic understanding links to research in the area of “personalized medicine”, “non-communicable diseases” and “big data” is important. The joint use and enrichment of existing cohorts including birth cohorts and bio banks is needed. The area of public health shall focus specifically on testing current and future preventive measures and shall answer the question: How effective are the implemented preventive measures? There is a continued need to develop the methods and implement trials for providing the evidence base for prevention. This includes but is not limited to novel applications from eHealth and ICT based developments as well as health services or patient empowerment. The role of migrant and “invisible” populations such as the unemployed youth shall be emphasized and is considered an overall crosscutting issue. Their health in varying environments and social integration is viewed as critical for public health of European societies. It was stressed that for research in the area, attention to diseases conditions prevalent in these groups is needed.

#### *7 Active and healthy aging*

Active and healthy ageing was considered a high priority area by most, as seen by the feedback from the Program Committee. The advisory group recommended to focus on prevention strategies (including new business models) and connected care, strategies to enable faster adoption of new technology and care models, as well as research on the use of ICT/technologies to better support independent living. Another focus area was related to treatment of chronic disease and in general the need for connected care strategies, including investigating how big data and e-health can contribute. Finally research on ageing processes in general and enabling citizen to remain independent was also seen as important areas to strengthen.

### **HORIZONTAL themes**

#### *8 Big data*

Overall, the feedback received was very supportive of the big data theme. Several overlaps with the vertical themes as well as the three horizontal themes (eHealth, mHealth, ICT; Integration of care, and Environment and Health) were recognized thereby emphasizing the cross-cutting nature of big data. The feedback focused on the same themes and key words used also in the AG report, e.g. interoperability, standardization, multidisciplinary, role of research infrastructures, data protection and improved citizen/patient involvement. It was widely agreed that collection of reliable data, their integration, processing and interpretation through novel computational approaches will revolutionize

healthcare by improving clinical decision making and patient management, leading to faster and less variable results. What was not clearly expressed in the AG report is the fact that Big Data from biobanks, electronic health records, cohorts, registries etc. will also provide the evidence base for future public health research and policies. Suggestions were made to start pilot projects based on existing datasets in regions where such data is available. PC also emphasized the important role of Research Infrastructures in generation, processing, storage and distribution of data.

### *9 eHealth, mHealth and ICT*

Strong recognition of the promise of eHealth, mHealth and ICT as essential enablers for better care, both in terms of prevention and therapy was expressed. In order to unlock ICT's true potential, better data quality and liquidity are essential. Further research efforts are therefore required to achieve stronger interoperability, to establish a proper framework for data security & privacy protection and to enable inclusion of new data sources (e.g. genomics). PC experts complement that a) one should have a user-centric, rather than a technology-driven approach in overcoming the challenges, b) that policy makers, health professionals and patients should be involved in the design, c) that evidence of impact is mandatory, both at the level of the individual patient/citizen and at the level of public health. Caveats that were articulated: digital health should not lead to increased social inequalities in access to care (e.g. exclusion of the elderly or the less well-off). Both in the design and the scaling up of digital health solutions, the necessary attention is to be foreseen in terms of user education and change management (training of care professionals as well as patients).

### *10 Integration of Care*

Endorsement of the importance of better coordination between stakeholders in the extended care environment, i.e. beyond the walls of the hospital, inclusive of primary, community, specialty and social care and with the empowered patient at its center, both for prevention and therapy is central. In addition to proposed research efforts on essential technology challenges, PC experts also ask for attention to collaboration processes, to interaction models & governance between stakeholders and to their education. Moreover, the recommendation is made to treat the topic with a sufficiently broad scope: including social care, mental care, palliative care, pain management, care of de-institutionalized patients, etc. It is requested to establish a set of outcome indicators to assess the impact on individual patient health, well-being and quality of life, as well as on population health and on care economics. Finally, it is suggested to make the link between themes 8 (Big Data), 9 (eHealth, mHealth, ICT) and 10.

### *11 Environment and Health, Green Solutions and Sustainability including Climate Change*

The research in the area of the health impacts of green solutions such as energy efficient housing and e-mobility shall be considering a setting of comprehensively study healthy cities, healthy mobility and environmental health. Specifically, it was recommended to focus on indoor and outdoor exposures to air pollutants. In addition to focusing on health in old age in this setting, the environmental impacts from pregnancy through childhood and adult age shall be emphasized. Enrichment of existing cohorts by novel technologies is highly recommendable for addressing environment and health. A prime example is the novel biomedical approach of the exposome jointly with social, spatial and economic aspects. Great potential for interacting with research in the area of big data has been identified as part of the discussion.

### *12 Social Sciences and Humanities, integration, inequalities, migration and ethic*

Following feedback from stakeholders, the working group would like to re-emphasise the importance of consent, privacy and respect for dignity and autonomous decision making of citizens. This may be achieved to some degree by the meaningful inclusion of patients, patient advocates and patient group representatives in projects. While the long-term effects of treatment and survivorship of patients affected by chronic diseases remain very important, cognizance should also be taken of public health issues such as the cost-effectiveness of multidisciplinary and combined therapies/interventions.

### *13 Sex and gender differences in medicine*

Women and men have different sex and gender-related risks for developing certain conditions and respond differently to treatment. Scientific knowledge on the impact of sex and gender differences is now better known in the research sector. The time has come to address remaining barriers and move to a more systematic approach of implementation into research and clinical practice for the benefit of patients and at the same time contributes to the reduction of inequalities in health for women and men. It is important to develop more precise and tailored approaches and care by the inclusion and analysis of data by age sex and gender.

## **CROSS-CUTTING themes**

### *14 Commercialisation within “Health, Demographic Change and Well-being”*

Following feedback it was emphasized that commercialisation describes how to take the ideas from the publications and bring them into the lives of European people. Science has to go together with industry to find new ways of doing research. We need a mixed group of both scientist and all types of directors, industry representatives and others from organizations. There were comments on the need to see more prevention. People need to own their health.

Integrated finance describes how different tools are needed along the trajectory of taking finance from an idea to a product. Comments pointed to the fact that in the H2020 there is a lot more than research, such as looking at data privacy and interaction with the policy and industry. Concepts should deliver products that are affordable for the general public and focus on prevention and precision medicine. Additionally, concepts that collect information to give oversight should be invested in, for example, a collection of information around biomarkers from which products could be made. The themes should be determined but the ideas themselves should be left to the applicants who can then run them through test beds.

### *15 Encouraging stronger and successful involvement of EU-13*

Academics in EU-13 are being severely underpaid. Instead of doing research many capable persons choose other professions which often underutilize their skills. This results in a much lower number of researchers in EU-13 than in EU-15. In order to increase the participation of EU-13 in H2020 the method of defining the salaries for participants in H2020 from EU-13 should not refer to their current very low salary levels but should be based on more unified methods like e.g. Maria Curie Skłodowska rules. Moreover, an effective awareness campaign on H2020 is needed. This is because there is little knowledge in EU-13 about funding options within this program and the

program itself. Keeping the quality of the research should be priority and funding within H2020 should be distributed based on scientific excellence.

## **SUMMARY**

The stakeholder replies overall support the AG report and conclusion:

- Strategic priorities for the next years with flexibility the third year
- Continue some of the previous issues
- More focus on overall environment
- May be mainly eHealth, mHealth and active aging
- Big data solutions, cybersecurity and trust
- Putting the pillars together

The stakeholder comments and the discussion with the members of the Programme Committee have an important impact on the process towards developing the Advisory Group Report and Appendix into a future Work Programme for 2018-202. The Advisory Group, chaired by Liselotte Højgaard and Paulo Lisboa, wants to express a deep gratitude for your comments and support in this process.