Revision of the EU general pharmaceuticals legislation

Comments on the roadmap / inception impact assessment by the Institute for Quality and Efficiency in Healthcare (IQWiG)

IQWiG appreciates the opportunity to comment on the document "Combined Evaluation Roadmap / Inception Impact Assessment". Our comments are as follows:

Defining unmet needs

- IQWiG supports the suggestion to develop a common understanding of the notion of "unmet medical needs" together with public authorities responsible for health technology assessment, pricing and reimbursement.
- A joint definition of unmet needs could support pro-active measures aiming at needs-driven research and development.

Developing a robust evidence base for decision making at all levels

- If a revised pharmaceutical policy aims at improving access to medicines it needs to ensure that sufficient evidence for decision making at the health care system level is available at the point of market entry.
- Any decisions made by EMA that affect the evidence base available at the point of market entry have a direct impact on the ability of health care systems to make evidence based decisions within their remit. If, for example, EMA decides to develop regulatory pathways that accelerate approval of drugs based on limited evidence, this might impede comparison of new drugs versus available treatment options (including best supportive care) which is required to allow decision makers on the health care system level ensure high quality health care and to distribute funds into most efficient treatments in a given indication.
- The time period used for drug development should ensure collection of sufficient evidence for decision making both for the regulatory decision and for decision within the health care systems. This could be achieved by regulators defining evidence needs together with downstream decision makers or by a parallel route of definition of evidence requirements which nevertheless should be met by pre-approval development programmes.
- A mandatory requirement to conduct comparisons with existing best standards of care within the drug development programme should be introduced. Even if these comparisons are not determining the regulatory decision on risk/benefit of the individual new drug, they are required for decision making at the health care system level. This requirement could be based on the legislation for cooperation in health technology assessment at a European level.

Making use of new developments in science

- Recent developments in science have resulted in smaller patient populations in some diseases. This requires more efficient study designs. One of the newer options are adaptive randomised, controlled platform trials that allow to investigate several treatment options in one trial. Efficiencies include joint control groups and seamless inclusion of new treatment arms into existing study environments. This means that these trials should be run across individual drug development programmes. To maximise the feasibility of these trials, a

drug development model that fosters combination of development programmes of several drugs (potentially via an independent body) should be explored. This would be particularly important in the situation of parallel development of several candidates of a given drug class.

Making better use of available data

- To allow making better use of the data available from the drug development programmes, EMA (like FDA) should routinely require the submission of individual participant level data on clinical trials supporting its approval decisions. These data should be used to support a better understanding of available drugs and the development of new treatment options. This would include analyses across individual studies or interventions. Analyses of these data by a defined set of third-party organisations (e.g. HTA bodies) should also be possible.
- In general, we need more high quality evidence to support decision making. i.e. we need more, better and less costly randomised controlled trials. Efficiency and feasibility of randomised controlled trials should be improved by decreasing administrative burden, streamlining patient recruitment and data collection through innovative trials designs, e.g. by using data from clinical practice from registry-based randomised trials. The European DARWIN initiative should not be limited to collecting and analysing observational data but should be developed into an instrument for conducting randomised controlled trials. As such, DARWIN could support learning health care systems. Examples like the UK RECOVERY trial have demonstrated the potential and value of such approaches even in a pandemic situation. Developing DARWIN into an infrastructure also allowing interventional studies could support both drug development and treatment optimisation after approval.

Cologne, 27.04.2021