

### 3. Large Scale Applications RFP – Feb 2020

Large Scale Applications (LSA) cover the use of EQ-5D in large datasets, such as in quality registries (defined by various 'categories': condition, medical specialism, treatment, device, etc.), national health surveys, and large-scale multinational cohort studies. This type of use is coined 'use as PROM' or just 'PROMS'.

Routine data collection is often done with *multiple aims and different users* (e.g., clinicians, researchers, patients, and managers) in mind, each of which have specific perspectives and data requirements. Despite the EQ-5D being part of many national (e.g. Sweden, England) and international clinical registries, no clear strategy exists from registry holders, payers, and the EQ Group on the use of routinely collected EQ-5D data in these settings.

Increasingly stakeholders of registries are aware that 'use' is much more than data collection alone; 'use' implies that the registry holder knows how to extract/aggregate/present EQ-5D information (with other information) which fits in the working process or quality process of the user; the user in turn must know how to fit these data into the workflow/role the user has. And particular features of EQ-5D are more important in the clinical context: individual reliability, suitable cut-offs, the meaning of difference scores, the obtaining of valid pre-intervention or pre-registry values of EQ-5D.

Over the last few years, outcome-based health care improvement has emerged, resulting in the terms 'PROM' and 'PREM'. *PROM* (patient-reported outcome measure) refers to health measures which provide information on the patient's/client's health as reported by the patient/client. By contrast, *PREM* (patient-reported experience measure) refers to the service quality apart from its outcome impact, i.e. the quality of process of care, including both professional behavior and features like accessibility, thereby complimenting the PROM.

While EQ-5D is not a PREM, decisions on its incorporation in current quality systems are usually made in parallel.

The EQ-5D has proven to be a *good candidate* for routine PROM collection in several large-scale applications, including those in the UK, Sweden and Canada. Many strong features of the EQ-5D are valuable for PROM use, in particular its appealing descriptive system. However, many issues still await solutions and evidence. The science behind this (say 'PROMetrics') appears to be as multidisciplinary and technically demanding as the science behind e.g. various

valuation methods. Features, like the individual reliability of a level score, a sum score, or the utility score are important if EQ-5D data are to be used as part of quality control cycles. Similarly, using the EQ-5D for benchmark purposes (hospital, caregiver, region) creates the need for descriptive summary indicators (not necessarily preference driven) and change-over-time indicators, where we need more evidence on how experienced benefit relates to EQ-5D differences (previous work from a.o. I.Buchholz). Designers of automated quality systems using PROMs (including EQ-5D) ask for meaningful thresholds to alert users, or procedures to derive these in their context. Graphically appealing yet informative presentation formats for comparative data ('dashboards') are needed to communicate the results of PROM initiatives. The use of PROM numbers in individual care processes requires protocols/tools to correct for *personal scale distortion* ('response heterogeneity'), and *true background influence* ('case-mix' adjustment); the assumption that individual differences cancel out as is done in economic evaluations and randomized clinical trials is not usable here. Response heterogeneity correction, while not needed in RCTs for validity, still would be very useful to improve power.

One of the key challenges faced by the LSA group, and the EQ group more broadly, is *the 'battle' with competitor instruments*, especially the PROMIS generic instrument, and, potentially, the E-QALY. This challenge will become more evident with the widespread adoption of PROMs in routine practice, either stand-alone - as in many registries - or as part of outcome indicator sets (e.g., ICHOM).

*International authorities* set up report schemes on the health care systems of countries, which increasingly rest on PROMs and PREMs. For example, OECD health ministers have agreed to promote the routine collection of PROMs and PREMs. This international push for greater use of PROMs, together with the emerging field of competitor instruments raises a number of issues: how does the EQ-5D compare with other PROMs in the context of routine outcome measurement in terms of their measurement performance, clinical utility, and usefulness in supporting decision-making at various levels within the system? In the across-country context, new desirable properties emerge like the cross-walk capability to other measures (see APERSU-CIHI report). In data collection, the alignment with a PREM measure is relevant.

How can we develop *business models* which work in these environments (professional societies, hospitals, international governmental bodies) ? In particular now most use is free ? So far the EQ-business in financial terms rests for 99% on health economical evaluation of drugs, which is quite different in terms of *research formats* and *research leads*.

The mission of the EuroQol Group is to improve decisions by end-users and stakeholders about health and health care, either using the EQ-5D as part of an outcome measurement framework or as a single measure. The LSA WG regards the use of large-scale datasets, given the aforementioned developments, to be at the core of the mission. And the LSA WG, with the Research Foundation's management, looks into the business model of EQ-5D as PROM.

Given the above, the RFP includes two areas of interest.

### ***3.1. Routine use of EQ-5D as a PROM in large-scale applications***

Routinely collected EQ-5D data could be held by individual hospitals, clinics, primary care practices, regional or national health bodies, insurance organizations or patient registries. Beyond data collection, the conversion of these data into 'information' for decision-making and quality improvement is a challenge. This implies a shift in scientific attention to user-defined data-processing and data-presentation. We therefore are especially interested in the following research areas:

#### *Analysis and reporting of EQ-5D as a PROM.*

Analyzing and interpreting change in EQ-5D as a PROM has not been investigated thoroughly. Multiple questions exist, including: how do EQ-VAS scores relate to EQ-5D dimension scores over time, does response shift affect change data, what is the relationship between the EQ-5D (in a cross-section or as difference scores) and the dominant clinical measure in a particular area, what is the best choice of or updatable method to obtain thresholds for the level or change (e.g. MID) in health, and how can we transform day-to-day data or routinely collected data into measures-for-decisions/reflection.

In the analysis and interpretation of EQ-5D data generated in this non-economic context, the index score as a summary score for the five dimensions may not be appropriate, particularly when these data are used to inform clinical decision making. As such, developing new scoring approaches to generate a summary score for the EQ-5D is imperative to advance the use of EQ-5D in these applications.

#### *Presentation of EQ-5D information for different stakeholders, for different purposes ('dashboards')*

While the EuroQol Group has made progress in the technical possibilities to compare EQ-5D data, the presentational formats that work best for the various user situations (e.g. for clinicians, decision makers, etc.) remain unclear. 'Dashboards' includes dynamic IT-features

like the option of pointing to results in graphs or tables, which then are shown in more detail. We need dynamic templates that combine EQ-5D descriptive, utility and EQ-VAS information for distinct modes of PROM use. Empirical evidence on best techniques could translate into an EQ-toolbox for PROM use. Better presentational formats may also help the business case for EQ-5D compared to other competitor instruments, if we create opportunities to capitalize on this added value.

Several options exist on case-mix adjustment (table-wise, regression-wise), where one ongoing study addresses case-mix adjustment on other EQ-outcomes than the utility score.

*The conversion of EQ-5D (and other) registry data into clinical decision tools.*

Such tools are a.o. used to support shared decision-making whereby patients who consider receiving a specific treatment A are presented with information on their relative position now (in EQ-5D terms) and on their expected future health based on their personal characteristics (which may include response style, and decision style). These developments capitalize on routine data collected in large-scale applications. While studies have created pilot tools, real world implementation studies are still lacking.

*Comparative performance*

Comparative performance of the EQ-5D as a PROM compared to other leading instruments being used in routine outcome measurement (e.g., PROMIS). This may include research into conversion/cross-walking which can result in asymmetrical feasibility (A can be projected on B, but B not easy on A). An initial study is present, but there is scope for extensions.

*Reporting behaviour, or 'response heterogeneity' or response style (RS).*

This is a systematic response tendency of a person, in terms of the scale used, unrelated to the 'true' health level. A commonly known tendency in elderly respondents is to avoid extremes. In various applications of EQ-5D in the context routine PROM measurement and in the assessment of population health, the detection and adjustment of response heterogeneity is vital. One study has been funded to investigate the presence of RS, but research is also needed to develop methods for reduction/correction of RS.

**3.2. Use of EQ-5D in studying inequalities in health at the clinical and population level**

We are interested in studies that measure inequalities in health and health care, using the EQ-5D, including the relationship of inequalities to social and individual determinants of health,

and specific policies (local, national, international). If the EQ-5D is contrasted with other measures of morbidity, properties of the EQ-5D might be explored in this context.

Another area of interest is population health, including changes in population health status, population risk factor models and population norms. We are interested in factors that determine population health status defined in EQ-5D terms, including demographic, cultural, epidemiological geographical and temporal factors, and disease impact/burden of disease initiatives.

Preferably research applications relate to the Agenda of the Health Inequalities Special Interest Group (info: Prof Erika Lubetkin). Currently at least one study explores the application of a large set of epidemiological and economic inequality measures with more to come.

### **3.3. Other**

This list does not exclude other research questions that may have specific merits, and can be proposed, bearing in mind the rationale of the LSA. For advice or guidance on developing your research proposal, please feel free to contact the co-chairs of the LSA WG (info listed at the end of this document).