D2.1 short report of existing patient engagement practices and processes

777450 - PARADIGM

Patients Active in Research and Dialogues

for an Improved Generation of Medicines

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1. Executive Summary

The overarching mission of the PARADIGM consortium is to provide a framework that allows structured, meaningful, sustainable and ethical patient engagement (PE) throughout three key decision-making points of the development of medicinal products: Research priority setting, Design of clinical trials and, Early dialogues with regulators and health technology assessment (HTA) bodies.

Drawing upon key PE initiatives consolidated from consortium partners, the overall aim of work package 2 (WP2) is to undertake a gap analysis (i.e. the comparison of actual performance with potential or desired performance) in order to provide an inventory of gaps across existing PE practices (that include frameworks, guidance, guidelines) and processes (that include protocols, methods, tools or templates), along with case studies that are relevant to patient engagement initiatives. The desired performance that WP2 is measuring against will be largely based according to the stakeholder needs, expectations and preferences that have been identified from the literature, survey and focus groups, and three Delphi methodologies conducted (see appendix).

The purpose of this document is to provide an interim report which represents a high level overview of the analysis to date around existing PE practices and processes, combined with some of the already known gaps in PE based on anecdotal and experiential evidence, which will be integrated into the first iteration of the tool being developed for gap analysis. This interim information is not yet designed to be exhaustive or conclusive as to the status of PE in medicines development. Rather it represents a snapshot of PE in medicines development with a focus on the 3 decision making points addressed by the consortium. Further interrogation of the initiatives identified here will be undertaken during the next stages of work to identify in detail where gaps exist, why the gaps might exist, and what could be developed (e.g. new guidance, tools and methods) in order to enhance and sustain impactful patient engagement.

We define here the types of PE initiatives that we aim to include in a detailed analysis. Briefly, these form 3 levels of information in descending order of detail and applicability; Level 1 – Frameworks: guidance and guidelines, including those that may also contain additional tools embedded in them, Level 2: Processes – tools, standard operating procedures (SOP), methods, protocols and templates, and Level 3: Individual case studies that describe in part or wholly the PE activity start to finish. These levels were then applied to inclusion/exclusion criteria from an initially large list of PE initiatives identified from within the consortium. The criteria were; 1) Were patient(s) or patient groups directly engaged? 2) Is the PE activity part of a i) framework, guidance or guideline, ii) process, or iii) case study? and, 3) Does the framework/process/practice or case study cover 1 or more of the 3 decision-making points? To complement the descriptive analysis, a targeted sampling approach was also conducted to identify some of the key frameworks and guidance that originate from different stakeholder groups (industry, HCP, regulator, HTA and patient organisations) to identify common themes covered by all, and conversely help to indicate where some of the gaps may lie.

One hundred and sixty-five initiatives were subsequently identified that fulfilled our criteria for inclusion (see section 3.3). From this sample a majority covered the design of clinical trials (59%) with one fifth covering each of research priority setting (16%) and early dialogues with regulators and HTA (12%). Nearly three quarters (73%) covered the general adult population, with very few indicating that they included specialist populations, such as young people, people with dementia, or their carers (all ~5%). Of the published guidelines and defined processes (<30% of total) there are several good detailed examples that cover either, the entire medicine research and development (R&D) continuum and/or specific stakeholder groups. Some of the common themes addressed in guidance by different stakeholder were as follows; 1) Defining the objective of the planned interaction and/or areas of common interest (“shared purpose”), 2) Establishing/defining roles and responsibilities, 3) Ensuring transparency in all processes (publicly availability of who, what, when and finances), 4) Providing compensation for time/costs and help with logistical planning, 5) Building capacity and capability (for patient’s to be effective contributors and for stakeholders to engage effectively with patients), and 6)

Optimizing insight generation from patient experiences and knowledge of living with the disease.

Overall some guidance documents are supported with templates and tools and detailed methods to guide the implementation of the underlying principles – but many guidance documents lack this more holistic approach to implementation. Three quarters (76%) of initiatives are individual case studies that contain varying degrees of information on the process and outcomes of the PE activity. There is a general lack of published detail to those examples, with relatively few that specify the guidance, guidelines and tools used to carry out a PE activity, or the level to which patients were actually involved in a given PE activity.
At this stage some identified deficiencies (in guidance)[2] that emerged from the general PE landscape include the lack of direct link between guiding principles and the details of how to actually implement them, and the further logical link to additional tools and templates to support that implementation. Included in those gaps are the specifics on how to adapt guidelines to stakeholder needs, and in particular to vulnerable populations and specific decision-making points of research priority setting, and early dialogues.
In terms of processes, tools and templates, some additional gaps were identified where greater detail or applicability is needed that could in part or whole account for vulnerable populations, or EU member state structures and legal systems. These were: written agreements for engagement between stakeholders and patients/patient organisations that permit the creation of an equal partnership for all involved; detailed compensation recommendations and templates for ensuring fair and appropriate compensation or reimbursement; detailed policy rules on handling competing interests and conflict of interest statement templates; rules of procedure and tool(s) for identifying and connecting interested parties for PE activities (e.g. “matchmaking”). Matchmaking methods and processes are particularly noted as a gap for HTA bodies.

The interim results here are reflected in outputs elsewhere within the consortium (i.e. survey and focus group work that has been undertaken). At this stage only high level gaps in PE can be identified or implied. The granularity of where those gaps lie, on what level, and to what extent, are beyond the scope of this report. The next steps will be to integrate this new information to further develop a tool with which to better qualify and quantify known and unknown gaps. The inventory that will be eventually created will be able to expand upon the details of some of those known gaps, clarify further some of the gaps that exist that are being addressed in related work in other initiatives and where complementary work could continue, and gaps that are either unknown or poorly recognized. All of this new information can create a focal point for future consortium efforts to address.