THE MANIFESTO FOR PATIENT-DRIVEN MEDICAL INNOVATION

Patient organisations believe that their voice and engagement would make major, positive changes to priorities at several stages in the medical innovation process:

Basic research on the causes of diseases: failure to make progress in many areas results from a lack of understanding of the causes of disease. Investments in disease research need to be more strategic with less duplication and a greater sharing of assets and data before there are likely to be new therapies. The natural history of many diseases, including most rare diseases, is poorly understood and this makes the development of effective interventions more time consuming and expensive than needs to be the case.

Benefits sought in new medicines: research is often 'push'-focused, targeted on what professionals in industry believe are easily measureable performance criteria; patients want a more 'pull' orientation, prioritising other, 'quality of life' and individually personalised measures that could point to different therapies (examples: non-motor symptoms of Parkinson's; wheelchair users' quality of life in muscular dystrophy).

Non-drug innovations: managing patients' lives in the later stages of disease requires technologies that need to be more actively developed, tested and reimbursed (example: non-invasive ventilation in Motor Neurone Disease). Sharing of knowledge and experience by patients and families promotes the effective development and application of these, and mechanisms to actively support this are needed.

Clinical research: there is a major opportunity in creating disease registers (with the support of patient organisations, working with Public Health England) and linking these to tissue banks (working with Medical Research Council, Technology Strategy Board etc). Tissue samples should be routinely collected in trials and be accessible to other researchers. There should be much more effort in establishing why medicines fail in Phase III and to investigate re-purposing of these products using these resources.

Regulatory benefit/risk assessments: these should take patients' views much more systematically into account and also reflect the increasing willingness to bear risk as disease progresses and treatment options narrow. Benefit/risk is disease-specific and so should be assessed by disease-specific panels which include input from those affected and/or their carers. Innovation is not just about 'cures' – interventions that improve life with condition are also highly valued by patients.

'Adaptive licensing': this early-access to medicines scheme needs to be progressed, with the key unsolved problems being how prices can reflect additional value proven after initial conditional licensing, and how patients can be supported to contribute the necessary "real world" data to demonstrate this.

Reimbursement decisions: regulators and payors should take account of forms of evidence besides classic randomised controlled trials (RCTs) and include qualitative factors going beyond the cost/quality-adjusted life year (QALY) to reflect the life situation of the patients and their families (example: use of thematic analysis and the experiences of patients and families living with the condition in question and their perceived value of the anticipated changes resulting from the intervention.).

Point of diagnosis: the moment and means of giving a diagnosis (particularly of a life-limiting disease) affects the subsequent, essential relationship between clinician and patient, and the latter's willingness to (e.g.) participate in research. Better support for clinicians on giving diagnoses and developing the doctor-patient relationship is needed. There also needs to be active encouragement for clinicians to be research active if patient willingness to participate is to be maximised.

Adherence: more research is needed into the psychological factors that lead patients (including those with life limiting diseases) to take inappropriate self-treatment strategies and 'drug holidays' and to understand and mitigate other forms of non-adherence to therapies of proven effectiveness. Furthermore, a better understanding of the wider social context of adherence is required.

Health Systems Uptake of Innovation: One of the major frustrations is the slow pace of innovation and change in the health and social care system, so as well as improving the research output, the challenge will be to create an environment conducive to truly sustainable innovation and service improvement.

Overall, all stakeholders, including academics, clinicians, industry regulators, reimbursement agencies and well-informed patients accessed through patient organisations need to work together to develop an 'influence guide' showing where and how their inputs would be most valuable in developing innovative interventions that respond to significant unmet needs in an effective, sustainable and patient friendly manner.

CASMI will play a leading role in developing the guide and promoting it in relevant forums of those who need to act upon its message.


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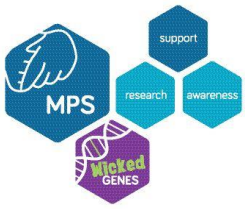
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