Patient-driven research is an imperative

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- From a moral point of view – because patients are the end-users of therapies and have a right to participate in research as partners
- From an instrumental point of view – because patients and society (payers) want innovation that brings added value – and this is only possible when patient perspective is fully integrated in research from the start
- One of the main reasons for “waste” in clinical research is when trials focus on research questions or measure outcomes that are not prioritised by patients (Chalmers et al, 2014)

Knowledge and practical value → economic value

Partnership with patients makes also economic sense

- Impact of a patient engagement activity that avoids one protocol amendment and improves enrolment, adherence, and retention is cumulative → increase in net present value of $62-65m, increase in expected net present value of $35-75m [1]
- NPV and ENPV [2] increase can exceed 500 x the investment in patient engagement → equivalent to accelerating product launch by 1.5-2.5 years (Levitan et al., 2017)
- Drugs developed using patient-centric designs recruit participants more quickly, and are more likely to be launched (87%) compared to other trials (68%) (Economist Intelligence Unit)

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[1] depending on whether trial is phase 2 or 3

[2] ENPV integrates key business drivers (cost, time, revenue, risk) into a summary metric for project strategy and portfolio decisions

Patient involvement in trial design adds value

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Patients bring unique knowledge and practical insights from experience

- Patients “always” offer unique, invaluable insights → their advice when designing, implementing and evaluating research “invariably” makes studies more effective, more credible, and often more cost-efficient (INVOLVE, 2009)
- Patient involvement improves outcome measures, recruitment (better recruitment strategy), retention (managing expectations), response rates, dissemination of findings (PatientPartner project, 2007)

\+ Increasing public confidence in clinical trials and appreciation of volunteers who participate in trials (EPF, 2011)

\+ *Patient Focussed Drug Development Reflection Paper* of ICH;

\+ *ICH E8* includes Patient Engagement as a key principle in that (section 2.3)

“HCPs see non-compliance, but patients can perceive poor communication, insufficient information or unhelpful attitude”

“In degenerative disease, not getting any worse may be equally valuable to getting better”