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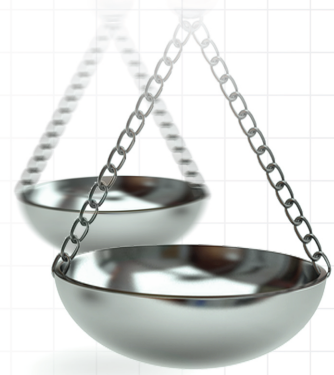

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Strengthening pharma's contract with society: the value of trusted partnerships between pharma and healthcare facilitated by real-world data

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This White Paper is authored by 11 industry real-world evidence (RWE) experts, with support from IQVIA, as part of the 'RWE Leadership Forum': a group of industry leaders who come together as noncompetitive partners to understand and respond to internal or external RWD/E challenges and opportunities with a single expert voice. Herein we aim to clarify the rules of engagement between pharma and healthcare in order to establish trust-based partnerships, which will unlock unique value for society, including the medical community and the ultimate beneficiary, the patient.

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A rare opportunity

As health systems find themselves under increasing pressure, the pharmaceutical industry has a unique opportunity to broaden the very basis of their engagement with health systems beyond predominantly one-dimensional relationships focused on access to innovative treatments. Instead, the pharmaceutical industry can look to build trusted partnerships around a common purpose of supporting the delivery of better patient outcomes through application of data-driven insights to improve treatment and care pathways, to advance medical practice and to optimize the utilization of healthcare resources.

The term real world data (RWD) describes data that are routinely collected within health systems on a patient's health status, in a purely observational fashion. RWD has the potential to play a critical role in establishing trusted partnerships: its very origin from within health systems, makes RWD particularly suited for generating evidence in a transparent and objective way through collaborations. As such, the generation of RWD can help foster trust and support new research partnerships that build a shared understanding around the intent of collecting data, the purpose this will serve, which trade-off decisions the data will inform and the value such partnerships offer to each party.

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The emergence of interdependencies

The traditional relationships between the pharmaceutical industry and healthcare systems are being fundamentally re-defined, by moving beyond typical transactional roles as seller and buyer, respectively.

As health systems struggle under increasing financial pressures, engagement on the sole basis of providing innovative treatments for payment makes it difficult for both parties to reconcile conflicting priorities. Establishing long-term, trust-based partnerships around broad, shared objectives such as aligning treatment around optimal patient outcomes, enables health systems and the pharmaceutical industry to solve problems jointly.

Health systems and the pharmaceutical industry recognize the value of evidence and insight generated by RWD: alongside governments, the pharmaceutical industry has fueled an increasingly rich RWD landscape, as some health systems lack the expertise or resources to generate insight from those data assets themselves.

Research-focused partnerships transcend the traditional monetization of data and aim to solve the challenge of sustainable innovation for better patient outcomes. However, in order to build these partnerships, trust must be established between both parties.

The value of trusted partnerships

The defining feature of trusted partnerships is the long-term pursuit of mutually beneficial objectives, in a transparent way, by combining complementary assets, resources and capabilities. As the home of deep scientific, clinical and analytical expertise, with an ability to draw on significant resources, the pharmaceutical industry has a lot to contribute to establish trust-based partnerships. In the case of trusted partnerships between health systems and the pharmaceutical industry, this common ground can be found at the intersection of innovation, efficiency and patient outcomes, with RWD facilitating transparency, objectivity and stakeholder engagement:

- Patient outcomes: achieving better patient outcomes in the real world is the ultimate prize for trusted partnerships between health systems and the pharmaceutical industry. At the very heart of this lies the optimization of treatment and care pathways;
- Innovation: long-term collaborations as part of a network of relationships, instead of one-off studies, can facilitate the build out of novel RWD infrastructure, which drives unique insights to advance medical practice;
- Efficiency: improving the operational performance of healthcare systems is a major driver of mutual benefit in trusted partnerships. Efficiency gains help to ensure the sustainable delivery of high-quality care and unlock funds for broadening patient access to cutting edge innovation.

However, there are important considerations for defining the common ground between health systems and the pharmaceutical industry, specifically, the issue of scale of such partnerships. We believe two complimentary modes of working should be considered:

- Scaling up from local pilots: establishing exemplars of best practice, by demonstrating the art of the possible locally first, for example, by partnering directly with one to two centers, and then transferring learnings across the wider health system;
- Driving structural change at (inter-)national level: partnering with national and international bodies, including regulators and payers, to drive change, for example, by furthering methodology and data standards.

Crucially, to reap the full benefits from optimizing along the dimensions of innovation, efficiency and patient outcomes, learning healthcare systems [1] need to embrace a virtuous cycle of continuous improvement and be open to international best practice sharing. While healthcare systems operate predominantly locally, the pharmaceutical industry with its global reach is well placed to facilitate such exchange of learnings across countries.

Implications for partnership models: from transactional to relationship building

Historically, many collaborations between healthcare systems and the pharmaceutical industry tended to be transient in nature and only on a need-to basis, with short-term funding and engagement commitments, and therefore they do not always support a long-term and sustainable, mutually beneficial partnership model. Short-term focus represents a major barrier to unlocking the unique value of novel RWD assets. Lead times of 5 years or more are common for such novel RWD assets to reach the requisite data volumes and levels of data maturity needed for high-quality evidence generation, well beyond the typical initial 2-year sourcing and curation period to secure data access; hence,

the importance of establishing long-term relationships that enable the sustainable collection of RWD by healthcare systems.

To move to trust-based partnerships, new models are required that ensure long-term commitment, transparency and joint ownership of insights, all in full compliance with data privacy requirements and ethical standards. Recognizing the need for combining the best available data from all partners to solve a common problem is a key factor for successful collaborations, such as public–private partnership projects [2,3], multi-stakeholder collaborations [4,5] or single company-led, multi-stakeholder collaborations [6–8].

Requirements for successful trusted partnership models

For trust-based partnership models to succeed, several barriers need to be overcome which requires a concerted, multi-stakeholder effort. Specifically, the following agreements and clarifications are needed from healthcare systems:

- Alignment on the rules and models for access and contribution to data:
  - Clarification of the value of improving the quality of healthcare data in the pursuit of delivering better patient outcomes, and communicate said value to patients in order to encourage sharing of their data for research purposes;
  - Clarification of 'research' definition to facilitate data sharing under preferential General Data Protection Regulation (GDPR) or Health Insurance Portability and Accountability Act (HIPAA) requirements;
  - Streamlined approval processes, for example, pre-agreed study archetypes, standard operating procedures (SOPs) for standard research approaches;
  - Accelerated approval of data linkage.
- Alignment on fit-for-purpose methodologies and suitability of data sources:
  - Clarification of how to control for bias, beyond solely relying on randomization;
  - Clarification of suitable data sources for assessing clinical effectiveness and safety, especially when relying on disease-specific intermediate outcomes rather than typically well documented final outcomes such as death.
- Alignment on governance and transparency, as pre-requisites for data access and patient advocacy:
  - Agreement on ethical redlines for research;
  - Agreement on the principles of assignment of responsibilities;
  - Agreement on ownership and acceptable use of deliverables, including publication rights.

Health systems, and patients, have much to gain from adopting those changes in order to facilitate trust-based partnerships with the pharmaceutical industry and to reap the full benefits they have to offer.

Commitment by the pharmaceutical industry

To create momentum in advancing the trusted partnership agenda, the authors of this commentary endorse the following principles. They have been designed to ensure fairness, transparency and, ultimately, maximize the value of such partnerships to healthcare.

- Responsible use of health data: we believe that health data should be handled responsibly, in adherence to data privacy and ethical standards. Where laws and regulations allow the re-use of health data for research purposes without specific consent, (for example, to promote public health), we believe that industry, academia and legislators have the joint responsibility to educate individuals and society about the significant benefits of appropriate data re-use. Individuals should have transparency over the use of their personal health data, therefore we are committed to publication of all studies evaluating the effectiveness and safety of interventions in public registries and timely publication of study results, ideally in peer-reviewed scientific journals;
- Balancing stakeholder interests: we believe that the digital age will transform many aspects of biomedical research, clinical practice and healthcare systems. To harvest the full benefit of these developments for patients and society, a balance must be struck between the individual's privacy rights, interests and autonomy over the use of their personal health data and the broader societal gains in obtaining new knowledge and advancing medicines. As perceptions on aspects of these debates vary broadly not only from country to country, but also between generations, this requires a long-term, inclusive societal debate. We are committed to be a transparent, pro-active stakeholder in this discourse, and we will publicly share our views on key aspects of the discussion;

- Multi-stakeholder collaborations: we believe multi-stakeholder collaborations, which aim to develop and implement responsible data sharing approaches, are an efficient, transparent and responsible way to facilitate access to (novel) health data for research and healthcare provision. These initiatives rely on a fit-for-purpose data model designed to ensure adequate data privacy and security, coupled with broad representation from different stakeholders and an independent Steering Committee that ensures strong governance and ethical use of health data. We are committed to support the adoption of such multi-stakeholder initiatives and welcome collaborations with a wide range of potential partners across multiple disease areas;
- Collaborative cross-industry approach: we believe the pharmaceutical industry should collaborate on nonproduct, disease area-specific evidence generation, to further expand on the foundations laid by evidence-based medicine [9] and contribute to the acceleration of medical practice. We commit to share the insights derived from RWD using the appropriate channels, to the extent permitted under the data license agreements, in order to improve the collective disease area understanding and empower patients to seek the right care, help healthcare professionals best treat their patients and support healthcare systems in preparing for future challenges;
- Long-term commitment: wherever possible and meaningful, we believe in supporting long-term commitments to the sustainable creation and curation of new data assets, including the routine capture of outcomes data, embedded within healthcare systems. Such long-term commitments ensure that innovation continues to live on and that value is shared with the very sources of the data collection effort, instead of being exclusively captured by the sponsor(s). The success [10] of such efforts is dependent on collaboration between multiple stakeholders, including electronic medical record providers;
- Frameworks and methodological standards: we support existing good practice for the generation of high-quality RWE, including ISPOR-ISPE guidance, for example, "*Good Practices for Real-World Data Studies of Treatment and/or Comparative Effectiveness*" [11]; "*Reporting to Improve Reproducibility and Facilitate Validity Assessment for Healthcare Database Studies*" [12] and "*Use of Electronic Health Record Data in Clinical Investigations*" [13]. Furthermore, we welcome collaborations with regulators and commit to engage in constructive dialogue on shaping frameworks for advancing the generation and acceptability of RWE in support of stakeholder decisions.

By endorsing these principles, we are paving the way for the pharmaceutical industry to place itself even further at the forefront as a trusted partner in delivering better outcomes for patients.

This document lays the foundations for establishing trusted partnerships between the pharmaceutical industry and health systems. Naturally, several practical questions remain such as how to ensure equitable investments in healthcare are made, where long-term value is not solely realized in a small number of specialist centers, but widely shared across the system, and what is the most appropriate mechanism with which to make an investment? Questions such as these deserve their own dedicated discussion in future papers.

The healthcare environment is ripe for embracing trust-based partnerships. Indeed, the long-term sustainability of a vibrant learning healthcare system depends on establishing trust-based partnerships which also involve the patient and medical communities. The pharmaceutical industry now has a rare opportunity to fundamentally re-define their relationships with health systems, healthcare providers and patients, with focus on reconciling innovation, efficiency and patient outcomes, with RWD in a facilitating role as the source of insight, transparency and objectivity.

By ensuring healthcare decisions, including both pharmacological and nonpharmacological interventions, are consistently informed by a growing body of high-quality evidence, those trust-based partnerships will fulfill their promise and unlock unique value for society, with benefits delivered to many stakeholders, including the medical community and the ultimate beneficiary, the patient.

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References

1. Friedman C, Rubin J, Brown J *et al.* Toward a science of learning systems: a research agenda for the high-functioning Learning Health System. *J. Am. Med. Inform. Assoc.* 22(1), 43–50 (2014).
2. Vaudano E. Public–private partnerships as enablers of progress in the fight against mental disorders: the example of the European Innovative Medicines Initiative. *Eur. Psychiatry* 50, 57–59 (2018).
3. Harmony-alliance.eu Algorithms To Transform Knowledge Into Better Medicine Outcomes. (2019). www.harmony-alliance.eu/en/news/wp7/algorithms-to-transform-knowledge-into-better-medicine-outcomes
4. Hripcsak G, Duke JD, Shah NH *et al.* Observational Health Data Sciences and Informatics (OHDSI): opportunities for observational researchers. *Stud. Health Technol. Inform.* 216, 574–578 (2015).
5. Miller R, Wong J. Using oncology real-world evidence for quality improvement and discovery: the case for ASCO's CancerLinQ. *Future Oncol.* 14(1), 5–8 (2018).
6. Durand-Zaleski I, Manley-Daumont M, Baas P *et al.* I-O Optimise: developing a unique multinational real-world evidence-based research platform in oncology. *Value Health* 20(9), A742–A743 (2017).
7. Garside J, Healy N, Besson H *et al.* PHEDRA: using real-world data to analyze treatment patterns and ibrutinib effectiveness in hematological malignancies. *J. Comp. Eff. Res.* 7(1), 29–38 (2018).
8. Catalyzing the Transformation of Healthcare Innovation (2019). https://newdigs.mit.edu/catalyzing-transformation-healthcare-innovation
9. A Brief History of Evidence-based Practice – Evidence Based Practice in Optometry EBP Australia UNSW (2019). www.eboptometry.com/content/optometry/article/brief-history-evidence-based-practice-0
10. Notice of Proposed Rulemaking to Improve the Interoperability of Health Information (2019). www.healthit.gov/topic/laws-regulation-and-policy/notice-proposed-rulemaking-improve-interoperability-health
11 . Berger M, Sox H, Willke R *et al.* Recommendations for Good Procedural Practices for Real-World Data Studies of Treatment Effectiveness and/or Comparative Effectiveness Designed to Inform Health Care Decisions: Report of the Joint ISPOR-ISPE Special Task Force on Real-World Evidence in Health Care Decision Making. *Value Health* 20(8), 1003–1008 (2017).
12 . Wang S, Schneeweiss S, Berger M *et al.* Reporting to improve reproducibility and facilitate validity assessment for healthcare Database Studies V1.0. *Value Health* 20(8), 1009–1022 (2017).
13. US Department of Health and Human Services Food and Drug Administration. Use of Electronic Health Record Data in Clinical Investigations, Guidance for Industry (2018). www.fda.gov/regulatory-information/search-fda-guidance-documents/use-electronic-health-record-data-clinical-investigations-guidance-industry