

Project SATURN – a Real-World Evidence and Data Collaboration with Existing European Datasets in Osteogenesis Imperfecta to Support Future Therapies

Sangiorgi L.^{1,3}, Boarini M.^{1,3}, Westerheim I.², Skarberg RT.³, Clancy J.⁴, Wang V.⁵, Mordenti M.^{1,3}

¹IRCCS Istituto Ortopedico Rizzoli, Bologna, Italy

²Osteogenesis Imperfecta Federation Europe, Heffen, Belgium

³ERN BOND, European Reference Network on Rare Bone Diseases, Bologna, Italy

⁴Mereo BioPharma Group plc, London, UK,

⁵UBC Late Stage, London, LON, UK

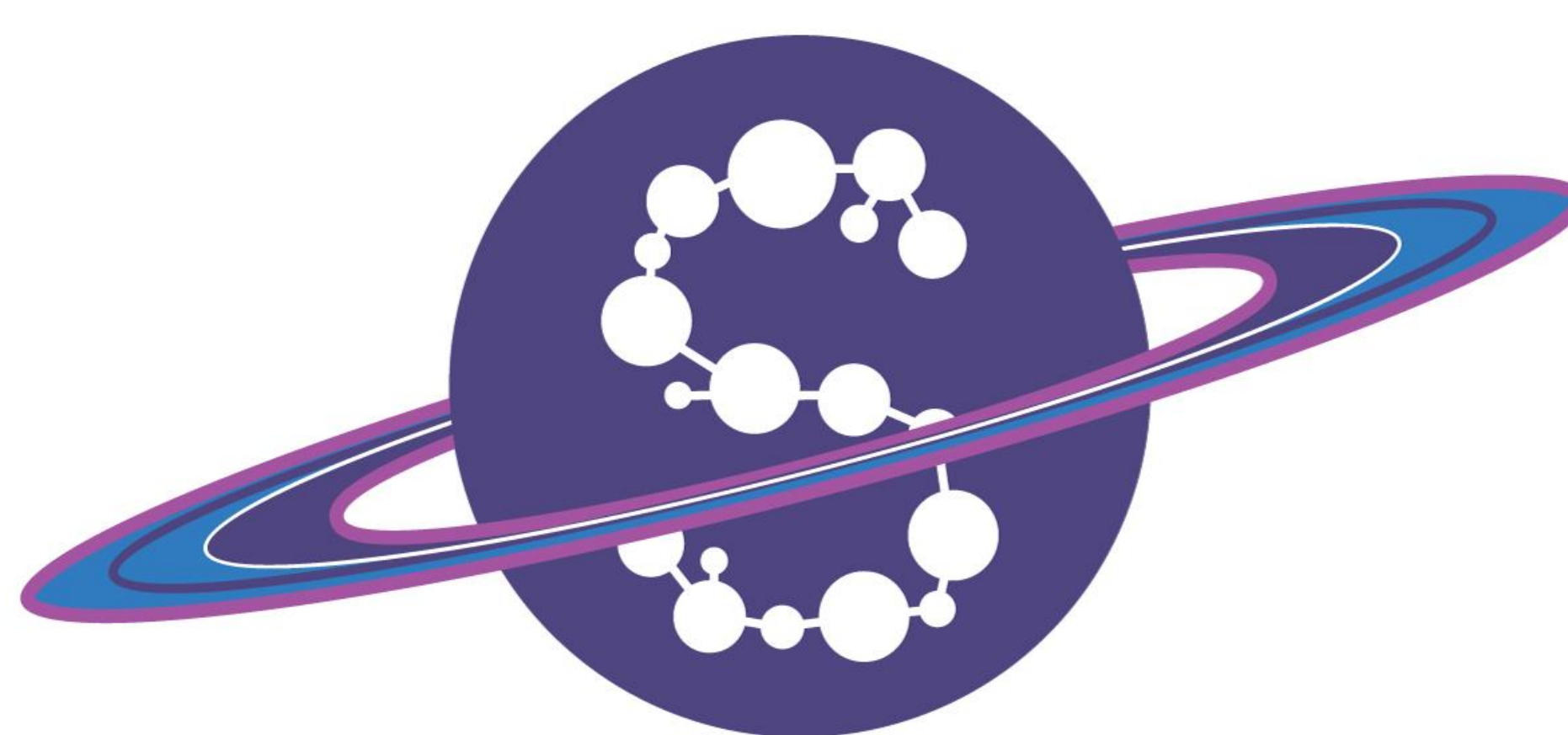
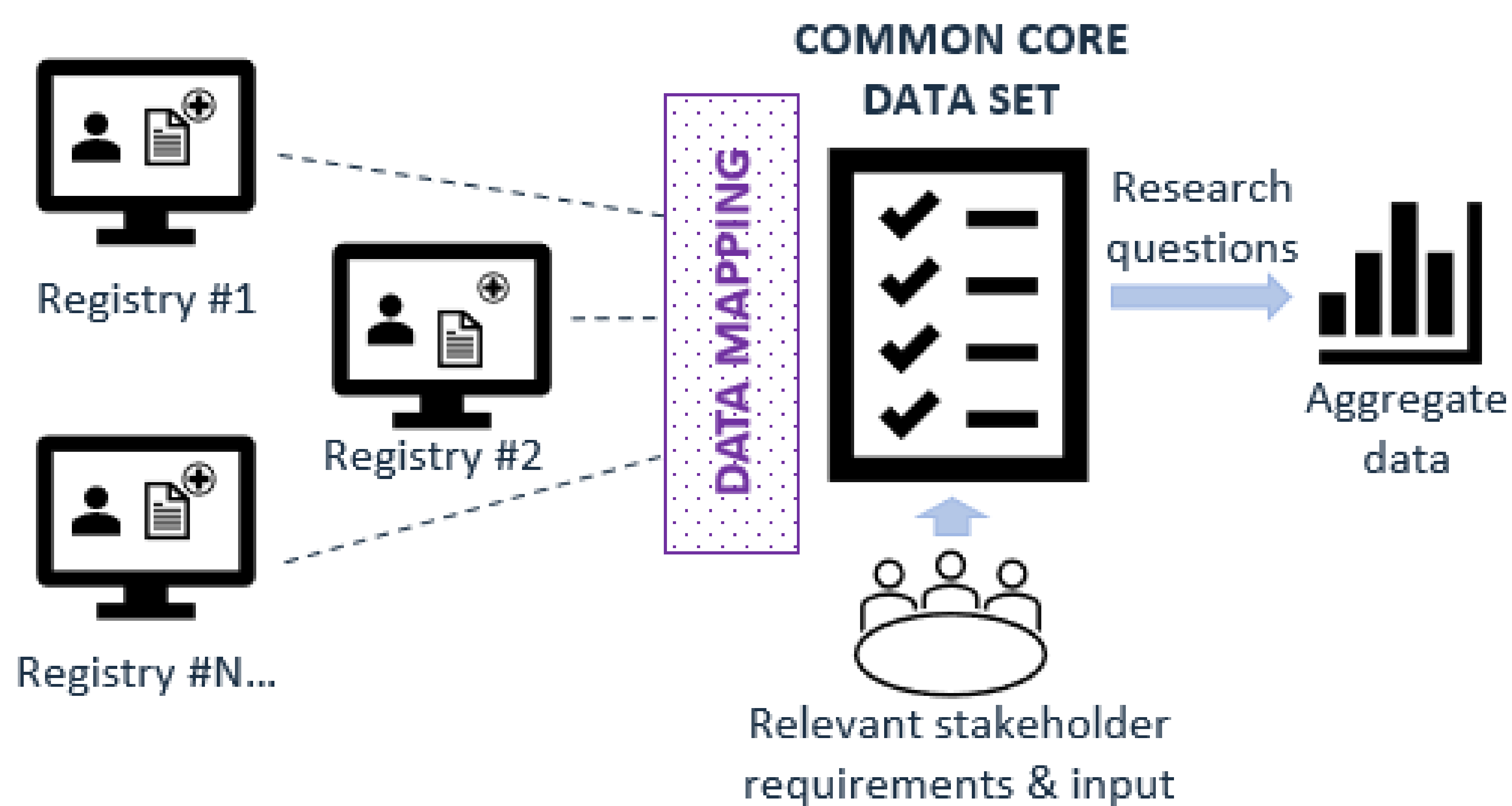
This paper describes an innovative initiative called Project SATURN: Systematic Accumulation of Treatment Practices and Utilization, Real World Evidence and Natural History Data in the Rare Condition Osteogenesis Imperfecta (OI). The objectives of this project are to generate a common core data set by utilising existing data sources to meet the needs of the various stakeholders and avoiding fragmentation through multiple approaches (e.g., a series of individual national requests/approaches, and unconnected with the regulators' potential requirements).

Regulatory marketing authorisation is not enough to ensure patient access to new medicinal products. Health Technology Assessment (HTA) bodies may require data on Effectiveness, Relative Effectiveness, and Cost-Effectiveness. Healthcare systems may require data on Clinical Utility, Savings, and Budget Impact. Furthermore, the exact requirements of these bodies vary country by country, and sometimes even region to region, resulting in a patchwork of different data requirements to achieve effective patient access reimbursement. In addition, clinicians require data to make informed clinical management decisions. This requirement is of key importance in rare diseases where there is often limited data and experience at the time of regulatory approval.

Ultimately, it is expected that such an approach will reduce the time to patient access to life-changing medications. Whilst Project SATURN applies to OI, it is anticipated that the principles could also be applied to other rare diseases and ultimately reduce the time to patient access to new medications.

Generate a common core data set by utilising existing data sources to meet the needs of the various stakeholders and avoiding fragmentation through multiple data collection approaches.

Same principles could be applied to other rare diseases and ultimately reduce the time for patient access to new medications.



Project SATURN
REAL WORLD EVIDENCE IN OI