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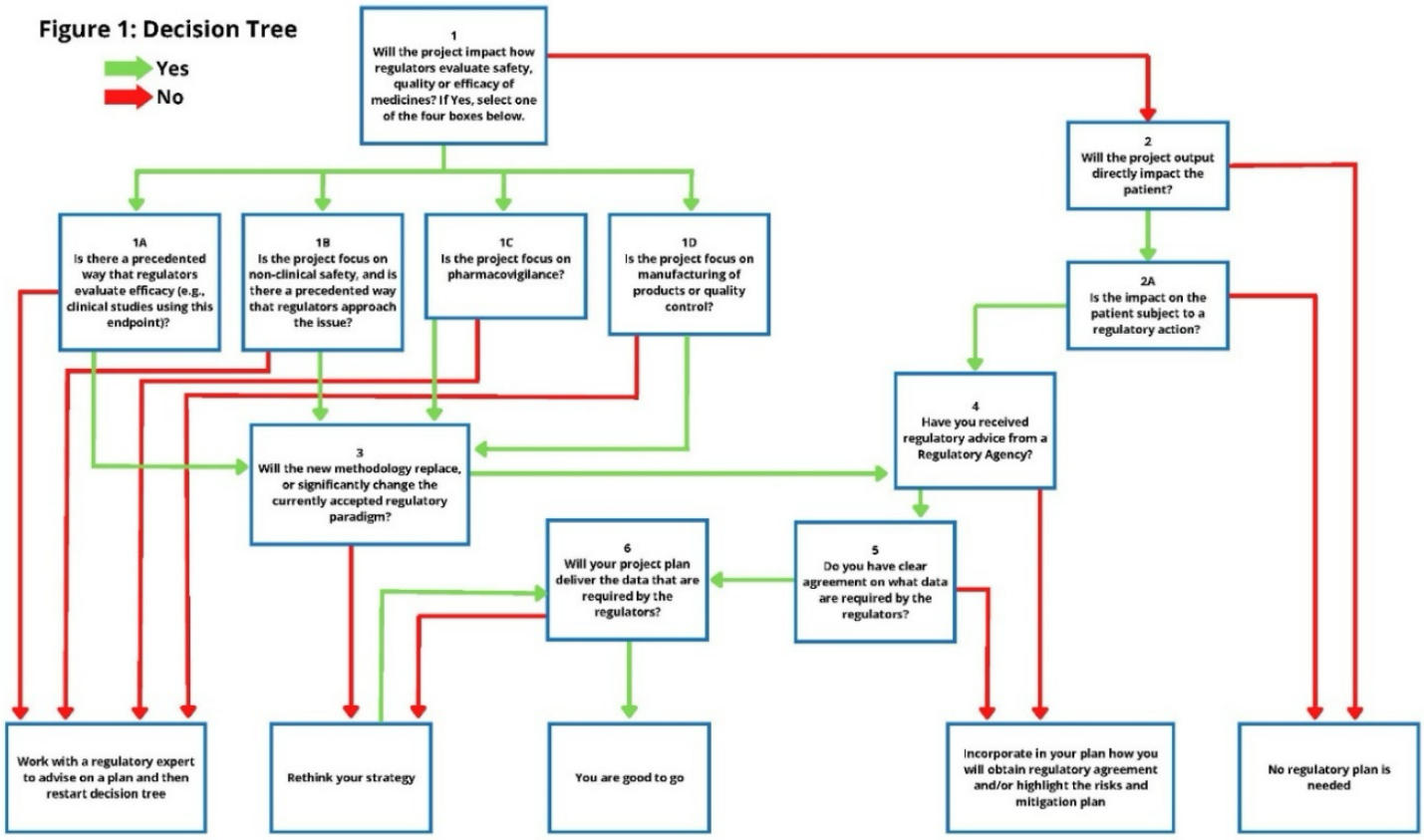
Supplementary information

Delivering regulatory impact from consortium-based projects

In the format provided by the authors and unedited

Supplementary Figure 1 | Illustrative decision tree to determine regulatory strategy and pathway
The illustrative decision tree referred to in the main article is provided below. Start at Box 1, and if your response to the question is "yes", ask yourself which of boxes 1a, 1b, 1c or 1d apply and then follow the green "yes" arrows or red "no" arrows as appropriate. If your response to the question in box 1 is "no" follow the red arrow to box 2 and proceed from there.



A selection of successful regulatory qualification procedures with the EMA and FDA, together with the associated links where full details of the regulatory process, and in some cases the development issues, is provided in Table 1. A full list of qualification of novel methodologies and drug development tools can be found on the respective regulatory agency websites:

- EMA: www.ema.europa.eu/en/qualification-novel-methodologies-medicine-development
- FDA: www.fda.gov/drugs/development-approval-process-drugs/drug-development-tool-ddt-qualification-programs

Supplementary Table 1 | Selected examples of successful qualification procedures

Year	Description	Link	Agency	Consortium
2024	Centiloid measure of Amyloid PET to quantify brain amyloid deposition	www.ema.europa.eu/en/documents/other/qualification-opinion-centiloid-measure-amyloid-pet-quantify-brain-amyloid-deposition_en.pdf	EMA	AMYPAD Innovative Health Initiative
2022	iBox Scoring System as a secondary efficacy endpoint in clinical trials investigating novel immunosuppressive medicines in kidney transplant patients	www.ema.europa.eu/en/documents/scientific-guideline/qualification-opinion-ibox-scoring-system-secondary-efficacy-endpoint-clinical-trials-investigating-novel-immunosuppressive-medicines-kidney-transplant-patients_en.pdf	EMA	Transplant Therapeutics Consortium Critical Path Institute
2022	Islet autoantibodies (AAs) as enrichment biomarkers for type 1 diabetes (T1D) prevention clinical trials	www.ema.europa.eu/en/documents/regulatory-procedural-guideline/qualification-opinion-islet-autoantibodies-aas-enrichment-biomarkers-type-1-diabetes-t1d-prevention-clinical-trials_en.pdf	EMA	Type 1 Diabetes Consortium Critical Path Institute
2022	Measurement of patients' perspectives through patient preference studies and incorporation into regulatory decision processes	www.ema.europa.eu/en/documents/regulatory-procedural-guideline/qualification-opinion-imi-prefer_en.pdf	EMA	PREFER Innovative Medicines Initiative
2020	Diary of irritable bowel syndrome symptoms - constipation (DIBSS-C)	www.fda.gov/media/145333/download?attachment	FDA	Patient-Reported Outcome Consortium Critical Path Institute
2020	Multiple sclerosis clinical outcome assessment (MSCOA)	www.ema.europa.eu/en/documents/other/qualification-opinion-multiple-sclerosis-clinical-outcome-assessment-mscoa_en.pdf	EMA	Multiple Sclerosis Outcome Assessments Consortium Critical Path Institute
2019	Asthma daytime symptom diary (ADSD) and asthma nighttime symptom diary (ANSD)	www.fda.gov/media/122726/download?attachment	FDA	Patient-Reported Outcome Consortium Critical Path Institute
2018	Proactive in COPD	www.ema.europa.eu/en/documents/regulatory-procedural-guideline/qualification-opinion-proactive-chronic-obstructive-pulmonary-disease-copd_en.pdf	EMA	PROactive Consortium Innovative Medicines Initiative
2018	Non-small cell lung cancer symptom assessment questionnaire (NSCLC-SAQ)	www.fda.gov/media/119250/download?attachment	FDA	Patient-Reported Outcome Consortium Critical Path Institute
2017	Dopamine transporter imaging as an enrichment biomarker for Parkinson's disease clinical trials in patients with early Parkinsonian symptoms	www.ema.europa.eu/en/documents/regulatory-procedural-guideline/qualification-opinion-dopamine-transporter-imaging-enrichment-biomarker-parkinsons-disease-clinical-trials-patients-early-parkinsonian-symptoms_en.pdf	EMA	Critical Path for Parkinson's Critical Path Institute
2017	Symptoms of major depressive disorder scale (SMDDS)	www.fda.gov/media/120615/download?attachment	FDA	Patient-Reported Outcome Consortium Critical Path Institute
2015	Total kidney volume (TKV) as a prognostic biomarker for use in clinical trials evaluating patients with autosomal dominant polycystic kidney disease (ADPKD)	www.ema.europa.eu/en/documents/regulatory-procedural-guideline/qualification-opinion-total-kidney-volume-tkv-prognostic-biomarker-use-clinical-trials-evaluating-patients-autosomal-dominant-polycystic-kidney-disease-adpkd_en.pdf https://force-dsc.my.site.com/ddt/s/ddt-project?ddtprojectid=85	EMA and FDA	Polycystic Kidney Disease Outcomes Consortium Critical Path Institute
2015	In-vitro hollow fiber system model of tuberculosis (HSF-TB)	www.ema.europa.eu/en/documents/regulatory-procedural-guideline/qualification-opinion-vitro-hollow-fibre-system-model-tuberculosis-hfs-tb_en.pdf	EMA	Critical Path to Tuberculosis Drug Regimens Consortium Critical Path Institute
2013	Novel data driven model of disease progression and trial evaluation in mild and moderate Alzheimer's disease	www.ema.europa.eu/en/documents/regulatory-procedural-guideline/qualification-opinion-novel-data-driven-model-disease-progression-and-trial-evaluation-mild-and-moderate-alzheimers-disease_en.pdf	EMA	Critical Path for Alzheimer's Disease Consortium Critical Path Institute