Need for Advanced Oncology RWE

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RWE—Where are we now?

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Real-World Evidence — Where Are We Now?

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Randomized, Interventional Study		Nonrandomized, Interventional Study	Nonrandomized, Noninterventional Study
Traditional randomized trial using RWD in planning	**Trial in clinical practice settings, with pragmatic elements**	**Externally controlled trial**	**Observational study**
RWD used to assess enrollment criteria and trial feasibility RWD used to support selection of trial sites	Selected outcomes identified using, e.g., health records data, claims data, or data from digital health technologies RCT conducted using, e.g., electronic case report forms for health records data or claims data	Single-group trial with external control group derived from RWD	Cohort study Case–control study Case–crossover study
	Generation of RWE		
Increasing reliance on RWD			

Reliance on RWD in Representative Types of Study Design.

RCT denotes randomized, controlled trial; RWD real-world data; and RWE real-world evidence.

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Global regulators call for international collaboration to integrate real-world evidence into regulatory decision-making

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News 22/07/2022

EMA has endorsed a joint statement calling for international collaboration to enable the generation and use of real-world evidence for regulatory decision-making published today by the International Coalition of Medicines Regulatory Authorities (ICMRA).

Key considerations:

- Whether the RWD are fit for use
- Whether the trial or study design used to generate RWE can provide adequate scientific evidence to answer or help answer the regulatory question
- Whether the study conduct meets FDA regulatory requirements

Rationale & Objective for Matched comparative data in NTRK gene fusion cancer in key tumor types

Victoria Study

ComparatiVe effectIveness study of real world ConTrOl of TRK positive cancer with patients from larotrectinib (Vitrakvi) clinical tRIAls https://clinicaltrials.gov//show/NCT05192642

Objective

- To generate comparative data / historical External Control Arm

Rationale

- RWE conducted two studies comparing patients with NTRK gene fusions who had not received TRK inhibitors vs patients without NTRK gene fusions to evaluate the prognostic impact of NTRK gene fusion. Results indicated a numerically higher risk of death in patients with *NTRK* gene fusions. *These studies were not designed for comparative purposes.*

Situation for Generating Comparative Data

NTRK+ patient identification requires data aggregation across several secondary data sources

It is difficult to find sufficient number of NTRK+ patients to allow for matching and outcome analysis across key tumor types

NTRK+ patient numbers drop dramatically when fusion is confirmed

No single data source contains enough patients in any tumor type

Pooling patient-level EMR/chart data available for purchase / partnering must still be supplemented with Bayer-sponsored chart review

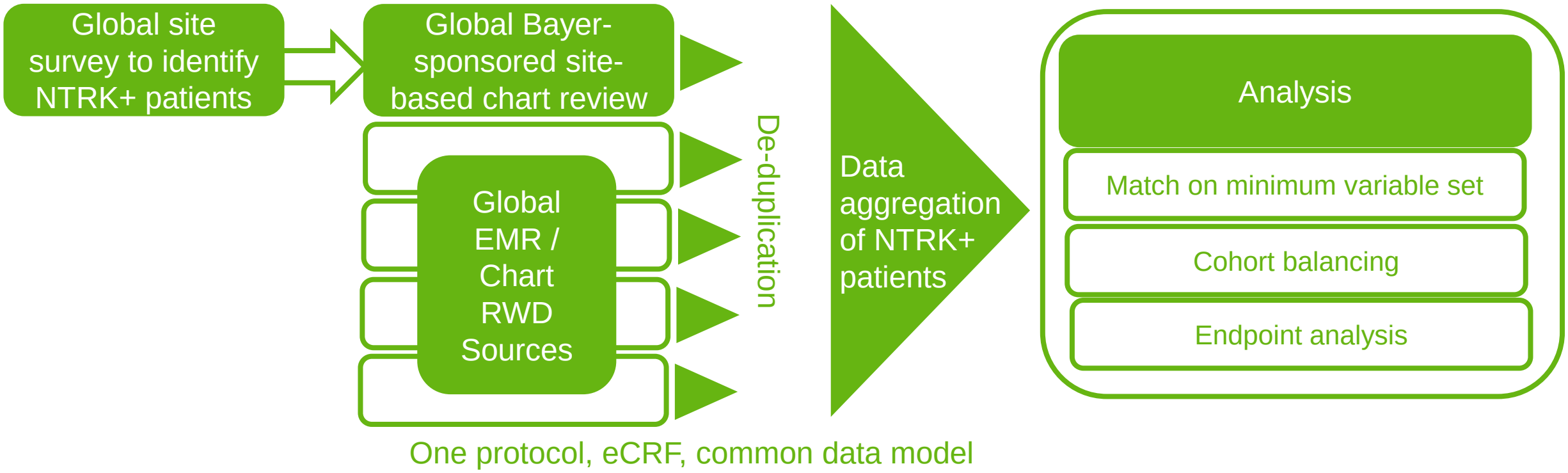
The potential for global chart review was assessed via survey to 14 countries and 800+ sites.

Patients from the US commercial sources are lacking.

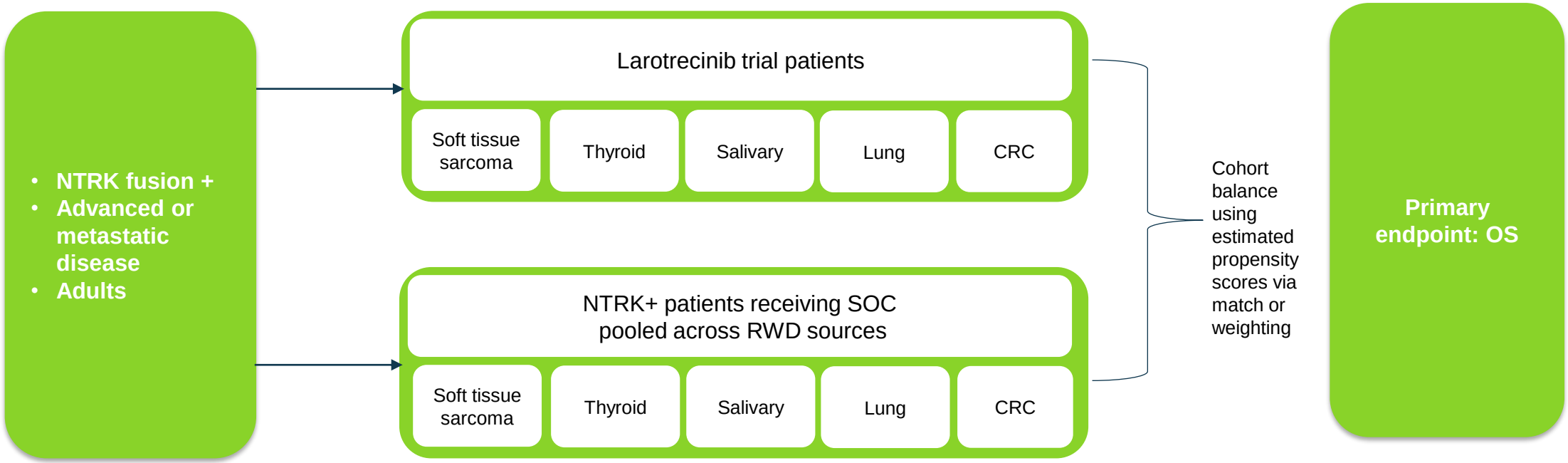
Several Bayer-sponsored observational studies underway/planned to further supplement the pooled data source with US patients

These studies are being designed to address different research questions

Proposed Data Aggregation for External Control Arm



Study Design



Potential Issues We Have to Consider with RW studies

- ✓ Designing a study that will meet multiple stakeholder needs
- ✓ Assess value of growing quality demands
- ✓ Balancing biases
- ✓ Internal education—RW studies are different from RCTs
- ✓ Contracting for all RWD sources
- ✓ Site review times
- ✓ Overall timelines
- ✓ Different definitions for study variables across pooled datasets
- ✓ Variable completeness

Please email me if you are interested in participating In VICTORIA!

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