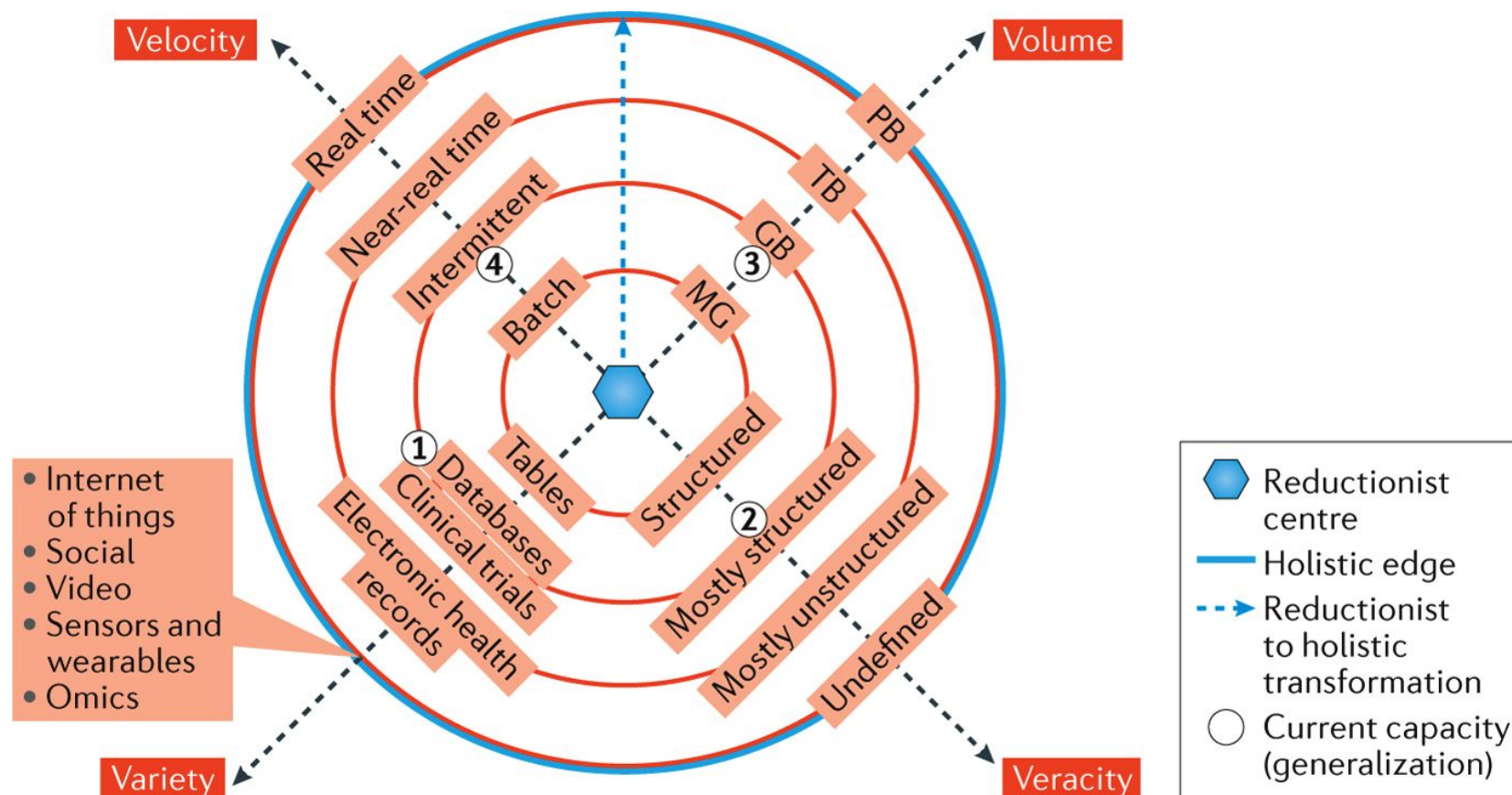# Digital Tools for Pivotal Trials

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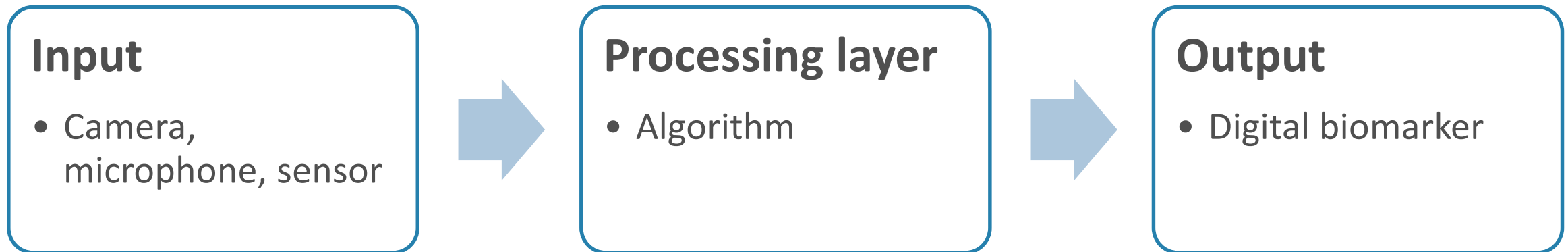# Digital tools in the expanding universe of big data

Nature Reviews | Drug Discovery

Khozin S, Kim, G, Pazdur, R. Nature Reviews Drug Discovery. 2017

Technical and procedural considerations

- Technical
  - Measurements
  - Verification
  - Validation

- Procedural
  - Clinical trials design and conduct

Technical and procedural considerations

- Technical

  - **Measurements**
  - Verification
  - Validation

Input	**Processing layer**	**Output**
• Camera, microphone, sensor	• Algorithm	• Digital biomarker

Coravos A, Khozin S, Mandl K. Nature Digital Medicine. 2019.

Technical and procedural considerations

- Technical

  - Measurements

  - **Verification**

  - Validation

Analytical verification

Uses engineering benchmarks to ensure the product is measuring and storing values accurately by confirming the tool's **accuracy**, **precision**, and **reliability**.

EXAMPLE: ability of heart rate sensor measuring electrical potential in millivolts to faithfully convert that signal into an accurate heart rate, expressed in beats per unit of time

Janssen | PHARMACEUTICAL COMPANIES OF Johnson&Johnson

Technical and procedural considerations

- Technical
  - Measurements
  - Verification
  - **Validation**

Validation addresses whether the measurement is **applicable** in the target population and **context of use**, which would render digital biomarker "fit for purpose"

EXAMPLE: heart rate as measured by sensor as an appropriate clinical endpoint in a trial, replacing tactile measurement
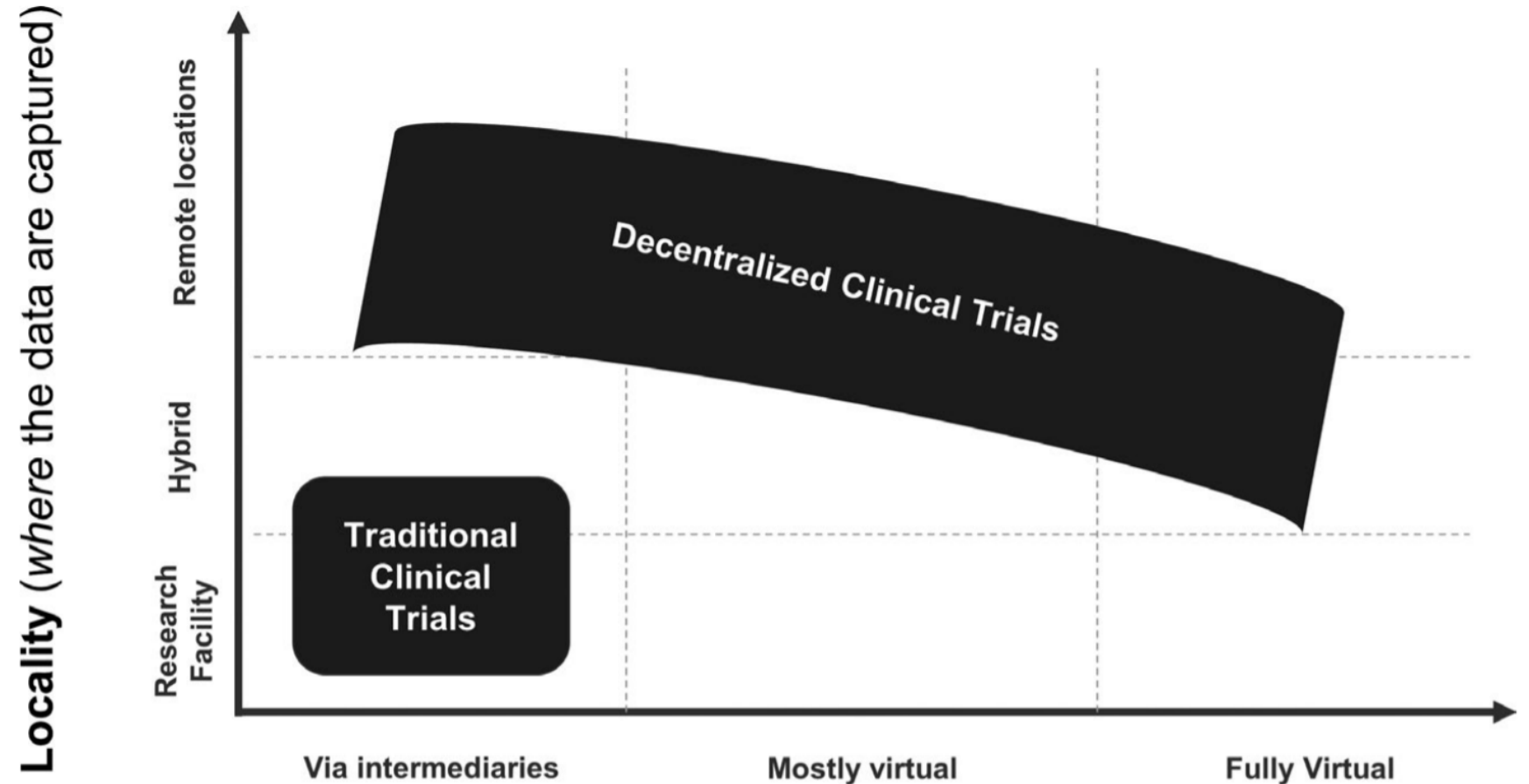
Technical and procedural considerations

- Procedural
  - **Clinical trial design and conduct**

Prospective clinical validation as part of a clinical trial

Technical and procedural considerations

- Procedural
  - **Clinical trial design and conduct**

Khozin S , Coravos A. Clinical Pharmacology and Therapeautics. 2019.

Thank You