

3<sup>rd</sup> Annual Congress on

# RARE DISEASES AND ORPHAN DRUGS

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## Developing novel, technology-derived endpoints for use in clinical trials

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**Statement of the Problem:** Mobile technology offers new ways to capture objective measurements as clinical trial participants go about their daily lives by utilizing novel endpoints, defined as new endpoints that have not previously been possible to assess or existing endpoints that can be measured in new and possibly better ways. These novel endpoints have the potential to provide high-quality data pertaining to outcomes that are meaningful to patients while enabling larger trials with reduced barriers to participation, making possible more sensitive, generalizable and patient-centric assessments. However, the pathway for selecting and developing technology-derived endpoints to support regulatory approval and labeling claims is not currently well-described.

**Methodology & Theoretical Orientation:** The Clinical Trials Transformation Initiative (CTTI) established a project team comprised of regulators, industry experts, academics and patient representatives. This team hosted workshops to write four use cases outlining the development of novel, technology-derived endpoints and conducted a systematic literature review.

**Findings:** CTTI proposes recommendations and tools to support the selection, development and application of novel mobile endpoints. In addition to the recommendations, tools include a novel endpoint development benefit framework; a selection tool to support decisions between viable novel endpoints for development; a guide to interacting with FDA; flowchart of steps for novel endpoint development; a supporting tool for these steps describing suggested approaches and considerations; use cases providing tangible examples of novel endpoint development (Parkinson's disease, diabetes, heart failure, Duchenne muscular dystrophy).

**Conclusion & Significance:** Novel, technology-derived endpoint development may be particularly valuable in rare disease research where there is significant unmet need for quality measures and where the possibility of hybrid or decentralized trials may facilitate recruitment that has been impossible to date. The CTTI recommendations and tools provide an important resource to support efforts to develop these endpoints and realize these benefits.

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