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# Evolution or Revolution:

## Translating PGHD into Measures that Matter, from Regulatory through Reimbursement

October 13, 2021
partner@evidation.com

# Webinar Housekeeping

- This session will be recorded.
- You will receive both the deck and a recording of the webinar tomorrow.
- We will be using a polling feature and encourage your participation.
- Please submit your questions for panelists using the Q&A feature.
  We will answer questions at the end of the session.

# Webinar Panelists

**Jennifer Goldsack**
CEO, Digital Medicine Society (DiMe)

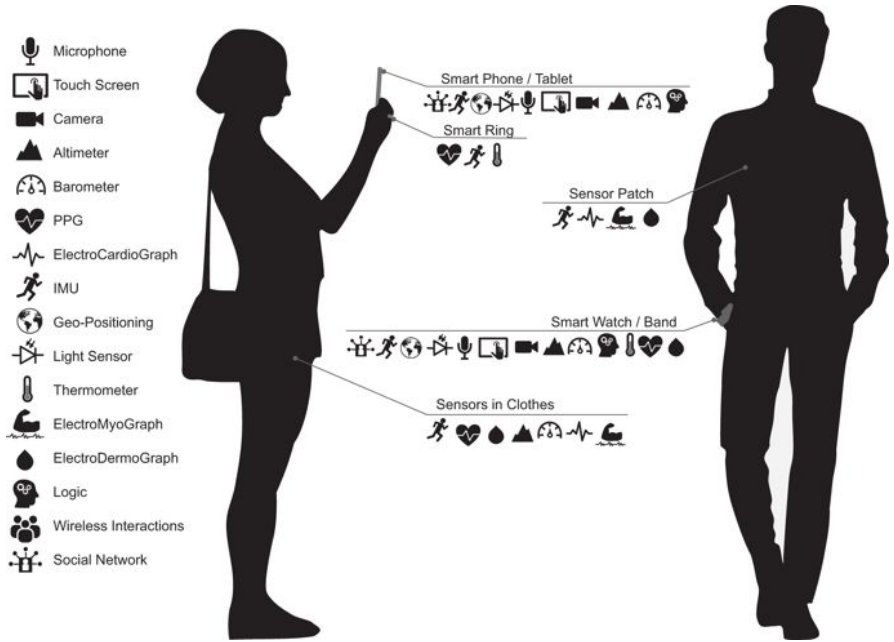
**Elise Berliner**
Global Vice President Innovation of RWE, Kantar Health

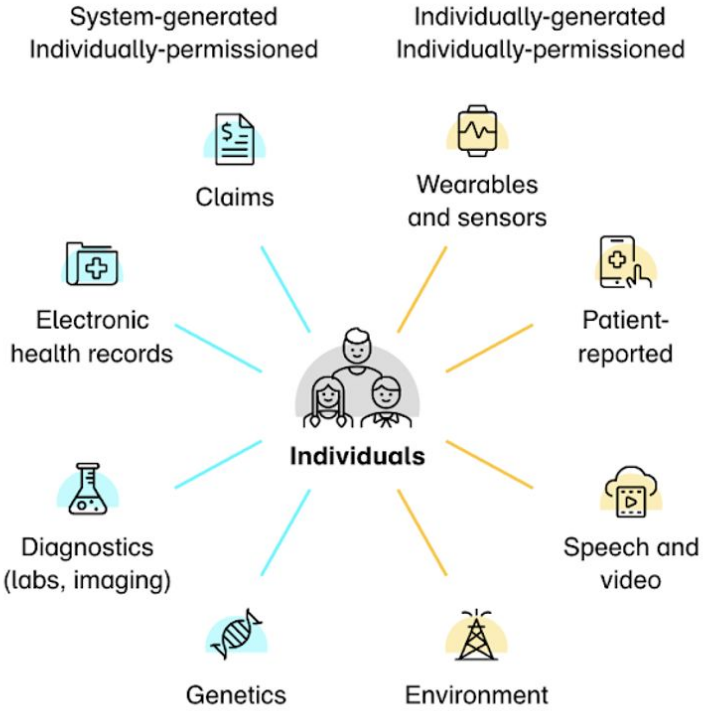
**Marco Prunotto**
Global Head of Technology and Translational Research, Roche

**Adaeze Enekwechi**
Operating Partner, Welsh, Carson, Anderson & Stowe ; Strategic Advisor, Evidation

**Bray Patrick-Lake**
Sr. Director, Strategic Partnerships, Evidation (Moderator)

# **Person-Generated Health Data (PGHD)** is wellness and/or health-related data created, recorded, or gathered by individuals for themselves (or by family members or others who care for an individual)

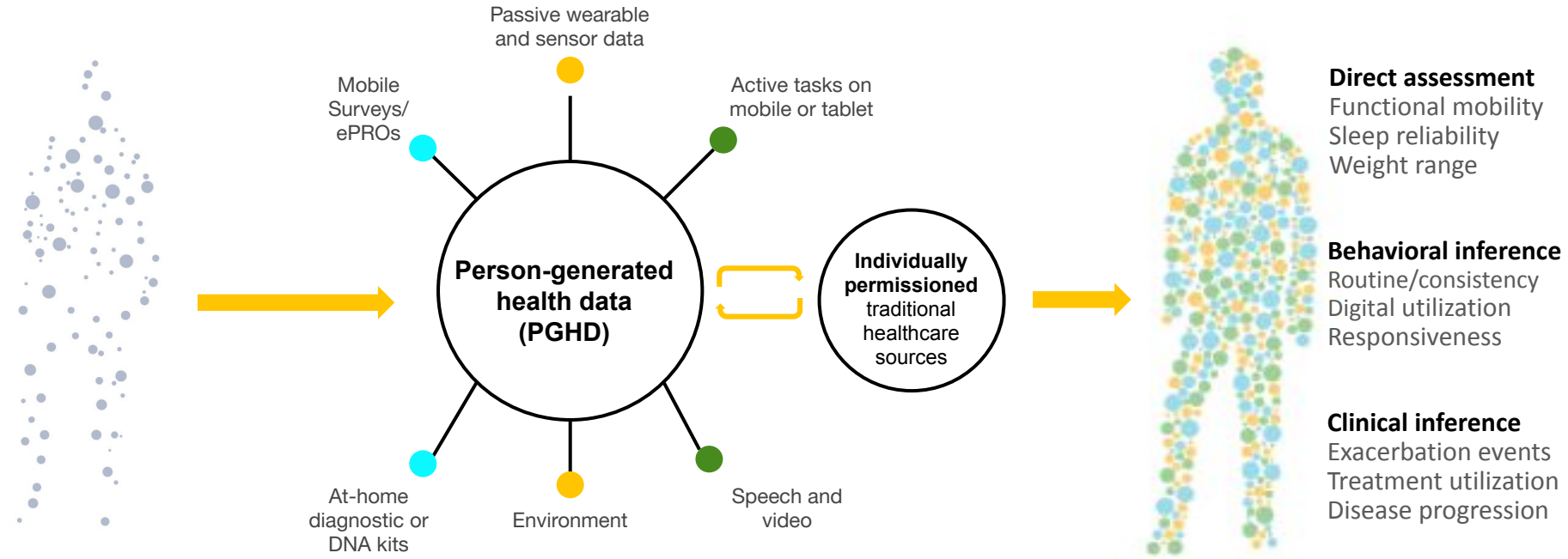





KOURTIS, LAMPROS ET AL., "DIGITAL BIOMARKERS FOR ALZHEIMER'S DISEASE: THE MOBILE/WEARABLE DEVICES OPPORTUNITY" NPJ DIGITAL MEDICINE, 2(1), 9. HTTPS://DOI.ORG/10.1038/S41746-019-0084-2; Definition: https://healthpolicy.duke.edu/sites/default/files/atoms/files/_determining_real-world_datas_fitness_for_use_and_the_role_of_reliability.pdf

# **Person-Generated Health Data (PGHD)** enables continuous monitoring of health outcomes at the individual level, to better understand and measure a person's experience with health and disease




Connect directly with participants

Consent per use

Return value to participants

Maintain connection over long period of time

**Poll:** Which best describes how your organization is currently implementing a PGHD strategy? Please select all that apply.

- Working with PGHD in an exploratory capacity
- Incorporating PROs into regulatory applications
- Incorporating digital endpoints in regulatory applications
- Submitting PGHD as evidence for reimbursement
- Supporting adoption of digital measures into clinical practice
- Curious but not ready

# DiMe Tour of Duty: The Playbook

## *Proof Points for Regulatory Openness to Digital Clinical Measures in Medical Product Development*

> "Compared to current standards for evaluation of the disease (MDS-UPDRS), DHT may offer new possibilities for more frequent objective measurements of the duration, severity and frequency of disease manifestations over time, that may provide more information than periodic clinic visits."
>
> *- Leonard Sacks & Elizabeth Kunkoski*
>
> doi: 10.3233/JPD-202416

> "Most notably, we're expanding the opportunities for digital health tools to become a part of drug review, to couple these capabilities to drug delivery to form a drug delivery system."
>
> *- Scott Gottlieb while FDA Commissioner*
>
> http://bit.ly/gottlieb-commissioner

> "To be able to harvest data remotely from patients has a tremendous value from a regulatory perspective."
>
> *- Leonard Sacks, FDA*
>
> http://bit.ly/sacks-FDA

> "There are different types of pain scales that have been used in clinical trials... maybe there's a better way of measuring it than filling out a form in front of a reviewer. Maybe they can do it in a pain app. Maybe they can use another type of technology."
>
> *- Chris Leptak, FDA*
>
> http://bit.ly/leptak

U.S. Food and Drug Administration & European Medicines Agency both participated in *The Playbook* Tours of Duty.



https://playbook.dimesociety.org/dossiers/pharma-exec/#micro-playbook-digital-clinical-measures-benefit-matrix

# DiMe's crowdsourced library of digital endpoints

## 69 Sponsors have collected digital endpoints

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*Primary, Secondary or Label Claim*

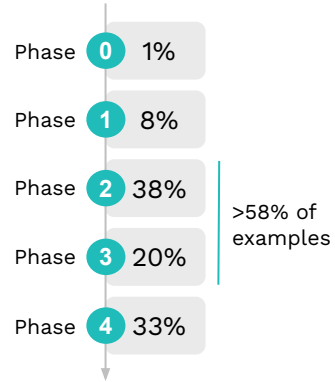
Abbott, abbvie, ABIDE THERAPEUTICS, ACTELION, ALTAVANT SCIENCES, ZOLL, ALKAHEST, Altec, ALTOIDA, aspen, AstraZeneca, AVAZIA, Takeda, Bellerophon, BAYER, Cavion, CuraSen, dexcom, dm, Daiichi-Sankyo, ucb, Eisai, gbt, GILEAD, gsk, janssen, Insulet, Forest Laboratories, Inc., VANDA, Genentech, LALLEMAND, Lilly, LMC, MEDICIS, VERTEX, MARREK, MERCK, NANTCELL, NOVARTIS, novo nordisk, OLATEC, ViiV healthcare, ottobock., PHARMANEX, Pfizer, PrecisionBiotics, proteus, PURDUE, SzeleSTIM, REGENERON, Sage Therapeutics, SANOFI, SENSIMED, SonVie, SANSUM DIABETES RESEARCH INSTITUTE, PRAXIS, MGH, Bright Cloud International Corp, SLEEPSPACE, EDIFICE, addex

*Exploratory Only*

CORTEXYME, Roche, sunovion, sonova, teva

### Sponsors start digital endpoint development early

*Digital Endpoints*

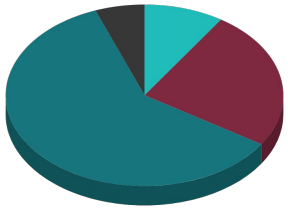



>58% of examples

*Only drug trials with reported phases are included

### Digital endpoints are being used across drug, device, and biologic development

*Investigational Product*



| | |
|---|---|
| Drug | 60.2% |
| Device | 24.7% |
| Biologic | 9.8% |
| Other | 5.3% |

### Pharma trusts digital products, primary/ secondary endpoints

*Endpoint Positioning*

| | |
|---|---|
| 63 | Primary endpoints |
| 148 | Secondary endpoints |
| 14 | Exploratory |
| 225 | **UNIQUE ENDPOINTS** |

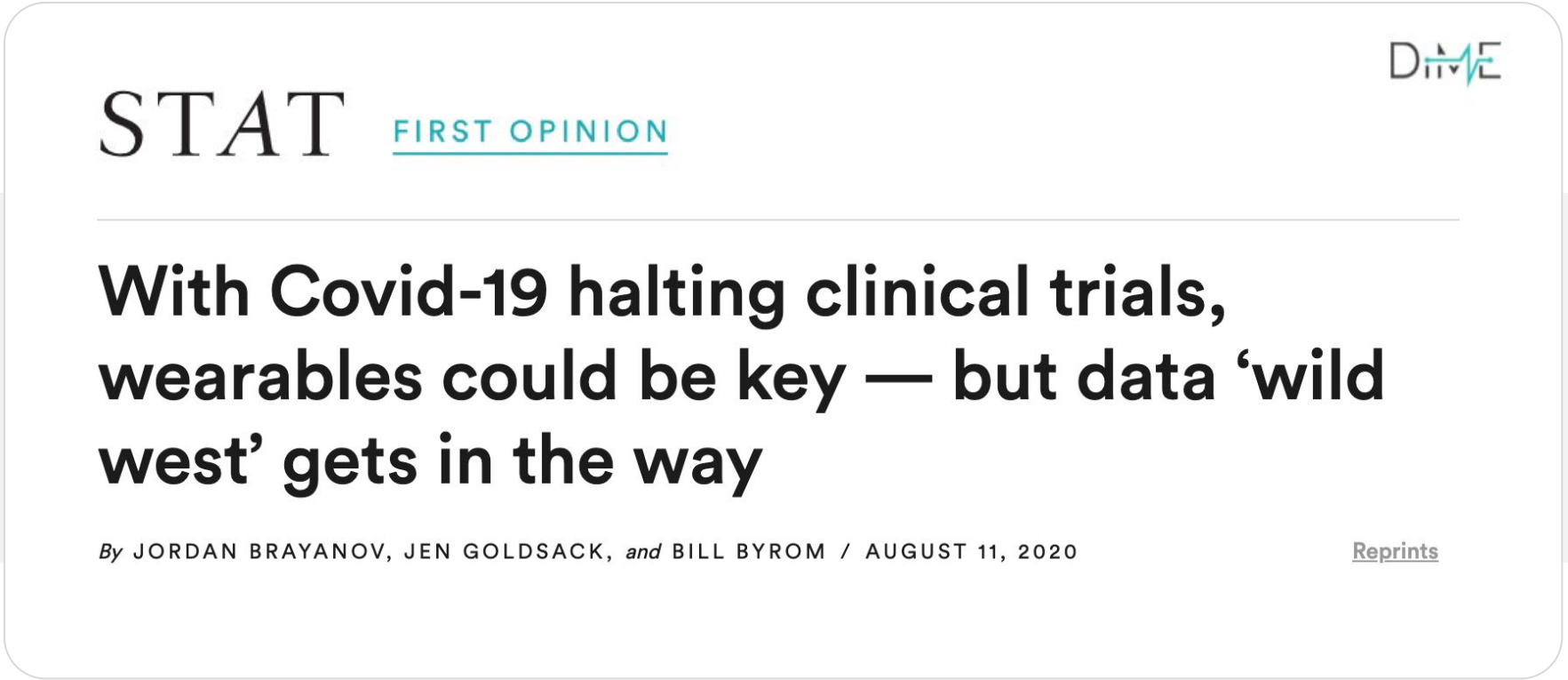
DiME DIGITAL MEDICINE SOCIETY

**Is your company's work missing?** Submit it to DiMe: https://bit.ly/DiMe-Endpoints

Source: https://www.dimesociety.org/index.php/knowledge-center/library-of-digital-endpoints

Last updated September 2, 2021

# Digital Medicine Society / Looking to the future of digital health measures



STAT FIRST OPINION

DiME

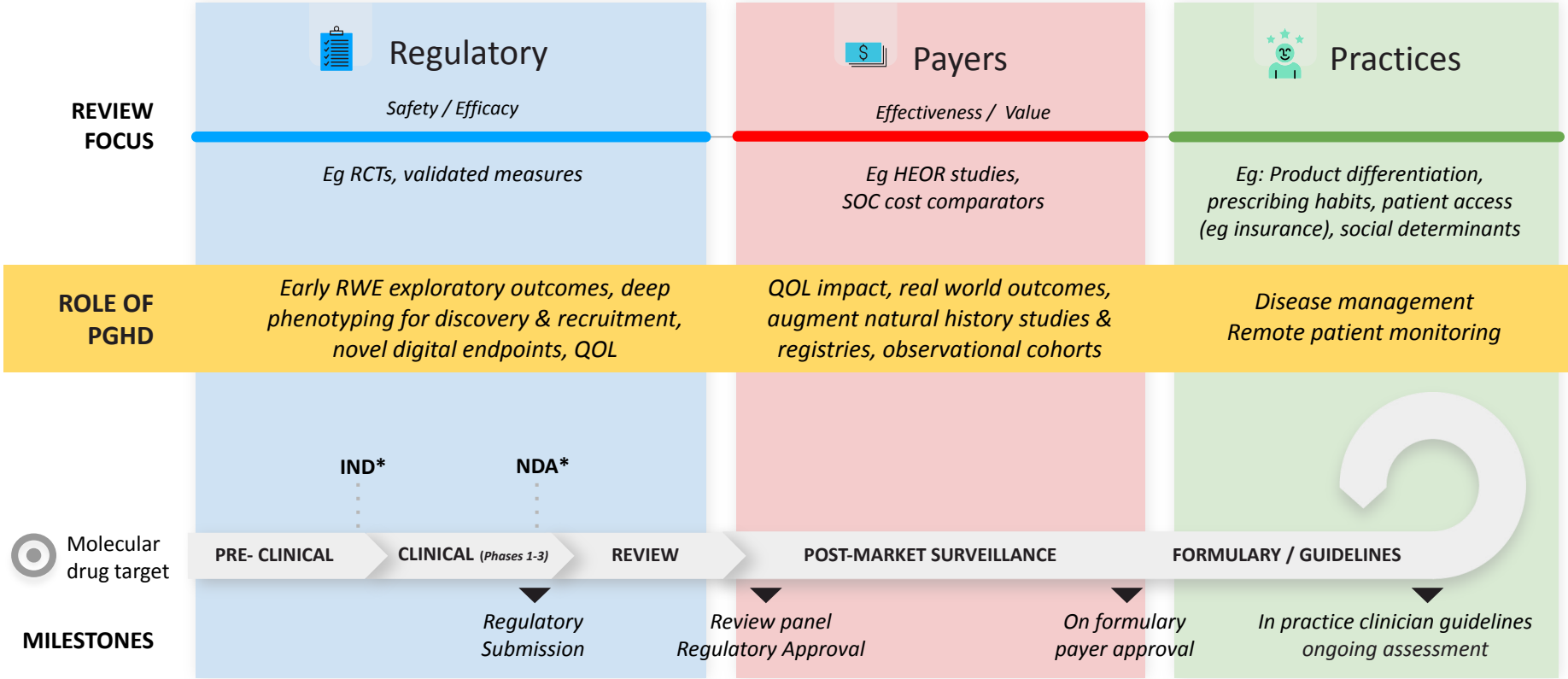
## With Covid-19 halting clinical trials, wearables could be key — but data 'wild west' gets in the way

*By* JORDAN BRAYANOV, JEN GOLDSACK, *and* BILL BYROM / AUGUST 11, 2020

Reprints

Source: https://www.statnews.com/2020/08/11/with-covid-19-halting-clinical-trials-wearables-could-be-key-but-data-wild-west-gets-in-the-way/

# Patient-centered drug approval to practice paradigm: The role of PGHD in understanding how patients **Feel, Function and Survive**






*IND = Investigational New Drug Application | NDA = New Drug Application*

# Ideal: Measuring What Matters

## Example: Heart Failure

https://connect.ichom.org/standard-sets/heart-failure/

ICHOM Core Outcome Set for Heart Failure


FDA guidance: “Treatment for Heart Failure: Endpoints for Drug Development Guidance for Industry” (https://www.fda.gov/regulatory-information/search-fda-guidance-documents/treatment-heart-failure-endpoints-drug-development-guidance-industry)

- “A drug that improves symptoms or function when added to standard of care would be valuable even if it did not improve survival or hospitalization. Moreover, it is possible that if a drug provided substantial and persistent improvement in symptoms or function, especially for patients with New York Heart Association Class III or IV heart failure, some decrease in survival would be acceptable.”



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# What is Typically Found in the Literature

Example: Technology Assessment on Retinal Prosthesis in the Medicare Population

74 outcome measures reported in 11 studies

- Only 3 outcome measures reported by 3 or more studies
- Only 4 outcome measures had evidence of validity and reliability
- Only 1 study directly measured QoL
  - The instrument was tested in a low vision population but may not be sensitive enough to measure changes in the RPS ultra-low vision population.
  - Several outcome measures have been developed specifically for patients with ultra-low vision but were not used in any study.



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# Developing Valid PGHD Measures:

Methodology:

- Qualitative research on what measures are important to patients
- Reliability: Does the PGHD measure results that are reproducible and internally consistent?
- Validity: Does the PGHD measure what it claims to measure?
- Responsiveness:  Does the questionnaire detect changes over time that matter to patients?

Patient Population:

- Is the PGHD tested on diverse populations with reporting of clinical and social determinants of health
- Patient selection bias based on patient health literacy and access to technology?

How to validate PGHD outcome measures in the context of RAPIDLY EVOLVING TECHNOLOGY



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# Use of PGHD in Clinical Care and for Quality Reporting

PGHD in Clinical Care: promising but more work needs to be done: https://effectivehealthcare.ahrq.gov/products/health-data-mapping/report

"Multiple randomized controlled trials (RCTs) have evaluated some PGHD technologies (e.g., pedometers, scales, BP monitors), particularly for obesity and hypertension, but health outcomes were generally underreported. We found evidence suggesting a possible positive effect of PGHD interventions on health outcomes for four chronic conditions. Lack of reporting of health outcomes and insufficient statistical power to assess these outcomes were the main reasons for "unclear" ratings. The majority of studies on PGHD technologies still focus on non-health-related outcomes. Future RCTs should focus on measurement of health outcomes. Furthermore, future RCTs should be designed to isolate the effect of the PGHD intervention from other components in a multicomponent intervention."

But CMS optimistic about the future of PGHD for quality reporting (2021 Proposed Rule: https://www.govinfo.gov/content/pkg/FR-2021-07-09/pdf/2021-14250.pdf)

"Many important data sources are not currently captured digitally, such as survey and PGHD. We intend to work innovate and broaden the digital data used across the quality measurement enterprise beyond the clinical EHR and administrative claims."



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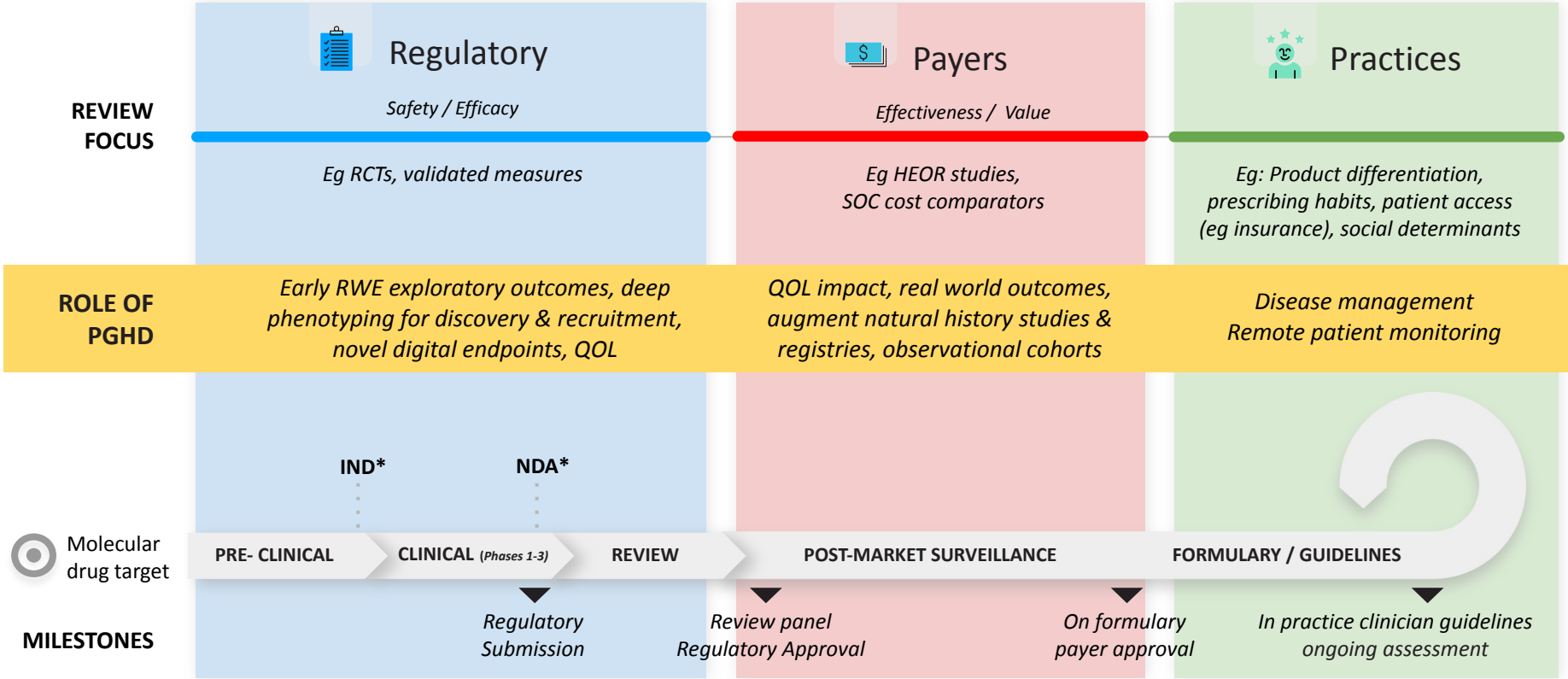
# Patient-centered drug approval to practice paradigm: The role of PGHD in understanding how patients **Feel, Function and Survive**



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Q&A
Email: partner@evidation.com

# APPENDIX

# Patient considerations that should drive digital measure selection and development

**CRITICAL PATIENT INPUT:**

## Meaningful Aspect of Health

Aspect of a disease that a patient a) does not want to become worse, b) wants to improve or c) wants to prevent

- *May be shared across some conditions and diseases*

What do you wish that you could do, but your condition prevents you from doing it?

What part of your life is most frustratingly impacted by your condition?

## Concept of Interest

Simplified or narrowed element that can be practically measured

- *Patients may have different symptoms*
- *Symptoms may vary over time*
- *Symptom relevance may vary over time*

What are the symptoms that most impact your ability to do these activities?

## Outcome to be measured

Specific measurable characteristics

- *Measures may be relevant to multiple symptoms*
- *Assess technical specifications of sensor and whether it is suitable for measuring this outcome in this population*

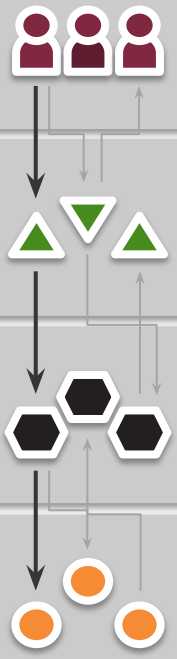
Do these measures make sense to you?

## Endpoint

Health research only; Precisely defined, statistically analyzed variables

- *Sensors may support multiple measures & endpoints*

How much change do we need to see in this symptom before it really starts to make a positive difference in your life?



This figure was adapted from original work by Evidation Health, with permission. This figure illustrates patient considerations that should drive digital measure selection and development. These should precede technical considerations..