

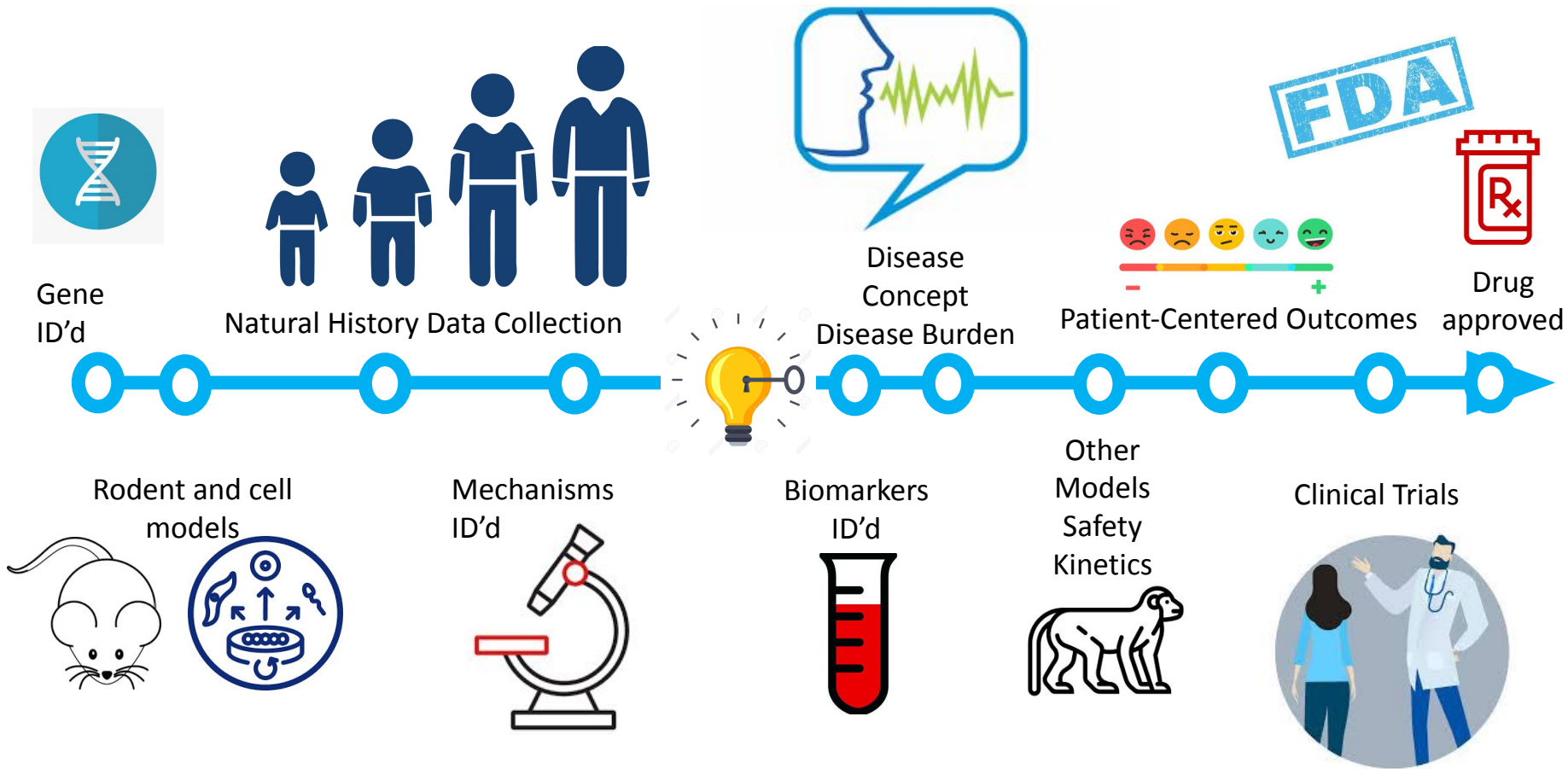
Advancing Outcome Measure for the Severely Affected

*Why collaborating is critical and
discussion about how to proceed*

11-11-21 Outcomes Workshop Introduction
JayEtta Hecker, Executive Director
DEE-P Connections

High Payoff of Phase I of DEE-P COMBINED Outcomes Project

- Built on culture of COMBINEDBrain and DEE-P - **COALITIONS** of PAGs collaborating to accelerate progress
- Completed comprehensive **LITERATURE** review and 1st draft **DISEASE CONCEPT** Map for severe DEEs following FDA guidance
- Laid foundation for collaboration of researchers leading innovative **APPROACHES WITH PROMISE** for severe DEEs; identified others
- Yielded a **BLUEPRINT** for a strategic approach to accelerate progress



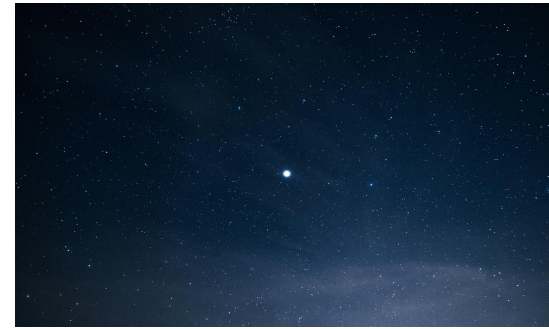
A strategic collaborative approach will yield high
payoffs for all



Payoffs of collaboration:

- **pools resources and intellect** to optimize success
- **maximizes efficiencies** - capitalizes on synergies; integrates data and learning
- **avoids fractured research**, which delays progress and limits continuous learning
- **enables a coordinated approach** which is more comprehensive, holistic, and robust.

Working together we can Focus on our North Star



- **Accelerate development** of potential new or adapted outcome measures/tools for severely affected populations
- **Champion tools** which reliably capture improvements for most severely affected populations
- **Fast track validation by FDA** by evaluating new tools as exploratory outcome measures in clinical trials



Next Steps

- **Collect and incorporate input on Strategic Plan**
- **Invite collaborators/co-authorship of manuscript** on outcomes and biomarkers for severely affected
- **Finalize sponsorships**, including funding formula, to underwrite collaborative efforts
- **Develop Priorities** for 2022
- **Convene regular meetings to enable collaborative decision making** and partnership among all stakeholders

Our Call to Action...



Breakout Discussion 1

Participants will be randomly assigned

15 minutes

Each group:

- Will have a designated **facilitator** and **notetaker**
 - to guide & capture the discussion
 - to provide feedback from the group to the full Plenary
- Aim to manage time so all participants can engage and each topic is covered

Core Topics:

1. What do you think are the **highest payoffs** of this expanded collaborative effort to advance outcome measures for those who are severely affected? e.g facilitating synergy amongst the 5 presented measures.
2. What **other (potential) measures** for severely affected do you think should be included in a broad strategic effort to accelerate progress?
3. What do you see as the **key opportunities and challenges** for pre-competitive projects which will accelerate progress toward validated outcome measures for those severely affected?

Report backs from first breakout discussions

Core Topics:

1. What do you think are the **highest payoffs** of this proposed collaborative effort to advance outcome measures for those who are severely affected? e.g facilitating synergy amongst the 5 presented measures.
2. What **other (potential) measures** for severely affected do you think should be considered for inclusion in a broad strategic effort to accelerate progress?
3. What do you see as the **key opportunities and challenges** for pre-competitive projects which will accelerate progress toward validated outcome measures for those severely affected?

Breakout Discussion 2

Participants will break out by primary role:

- PAGES
- Researchers
- Industry
- Clinicians

Those with “other” roles can select which group to participate in

15 minutes

Each group will have a designated **facilitator** to:

- Guide & capture the discussion
- Identify a **spokesperson** to provide feedback from the group to the full Plenary
- Manage time so all participants can engage and each topic is covered

Core Topics:

1. What do you see as the **key opportunities and challenges** for expanding the DEE-P/COMBINED partnership to accelerate progress toward validated outcome measures for those severely affected?
2. What do you see as **key next steps** to identify biomarkers and outcome measures for severely affected DEE's?
3. Do you see enough promise for payoff that you would **consider becoming a funding partner or active participant** to expand the DEE-P/COMBINED partnership?

Report backs from second breakout discussion

Core Topics:

1. What do you see as the **key opportunities and challenges** for an expanded DEE-P/COMBINED partnership to accelerate progress toward validated outcome measures for those severely affected?
2. What do you see as **key next steps** to identify biomarkers and outcome measures for severely affected DEE's?
3. Do you see enough promise for payoff that you would **consider becoming a funding partner** or active participant in the DEE-P/COMBINED partnership?