Return of Results
Aggregate and Individual

Jessica Scott, MD, JD
Head of R&D Patient Engagement, Takeda

David Leventhal
Senior Director
Clinical Innovation, Pfizer

March 18, 2019
Disclaimer
The views and opinions expressed in this presentation are those of the individual presenter and do not necessarily reflect the views of the Clinical Trials Transformation Initiative (CTTI) or the U.S. Food & Drug Administration (FDA).
Session III: Post-Trial Communication & Engagement

1. Evolving landscape
   • Overcoming challenges and progressing solutions
   • Working as part of multi-stakeholder consortia
   • Seeking to progress change internally and externally

2. Plain Language Summaries

3. Individual Return of Results
Takeda, Pfizer and others have been partnering over the past five years with various organizations including:

- Harvard Multi-Regional Clinical Trial Center
- TransCelerate BioPharma
- Health Research Authority Task Force on European Union Clinical Trial Regulation
- Layperson Summary Guidance
- Patient Data Access Initiative
- Supporting individual Public-Private Partnerships
Plain Language Summaries
Plain Language Summaries

- Make results accessible to study participants and general audience
- Aggregate results of a single trial written in plain language
- Explain technical terms and complex concepts in simple language

For the public
Plain language summaries

EU Clinical Trials Regulation 536/2014 (Article 37) (EU CT Regulation)

- New EU database once it becomes available
- Annex V ten elements that must be addressed in the lay summaries
- Consistency in the way trial results are presented will be helpful

Effective from 2020
Working Collaboratively

- Harvard MRCT*
  - MRCT Return of Results Guidance Document, Version 3.1, December, 2017
  - Return of Aggregate Results to Participants Toolkit Version 3.1

- TransCelerate BioPharma Inc**
  - Recommendations for Drafting Non-Promotional Lay Summaries of Clinical Trial Results EU CTR Task Force and formal Guidance
  - Layperson Summaries of Clinical Trials: An Implementation Guide

---

Remaining barriers/challenges

- Sponsors need to develop summaries and method of distribution
- Pre-publication concerns—no clear position from journals
- Need for clear FDA guidance
- Role of Independent Review Board (IRB)
- Potential to be seen as promotional

"84% of Investigator/Physicians agree aggregate results should be shared with patients → 44% have never shared with study participants”

Harvard MRCT Survey
Return of Individual Results

"I don't think it is just an opportunity - I think it is an obligation - an unmet obligation that pharma disseminate updates on the drug and on your trial.”

Patient, US
Understanding the landscape

TransCelerate Survey: Patient Perspective*

Over 3,000 patients surveyed across 36 countries - 2017

66% 'Knowing that my health and treatment record will be shared with me after my participation in the trial (e.g. my personal results)'

83% The majority of patients want their lab/test results

68% want to know whether they received study drug or placebo

Regulatory changes
EU Clinical Trials Regulation 536/2014 (Article 37) (EU CT Regulation)

Key legislative considerations
HIPAA, CLIA, GDPR, California Privacy legislation

*Accessed on March 15, 2019 at:
Return of individual results to participants

Consensus Study Report*
A landmark in the individual return of results space providing recommendations for the US, July 2018

Understanding the value of accessing Clinical Trial Data / Results

- Inform ongoing treatment
- Decrease expenses / risk of repeated tests
- Improved trust
- May improve clinical trials efficiency
- Portability of data
Gaining clarity on Individual Return of Results – what & when

- Harvard MRCT*
  - Return of Individual Results to Participants Recommendations Document Version 1.2 (Guidance), November 2017
  - MRCT Return of Individual Results to Participants Toolkit Version 1.2 (Toolkit), December 2017

A: Urgent Results & Urgent Incidental Findings

- Participant Screened
- Pt On Trial
- Pt Last Visit
- End Trial

B: Routine Results & Non-Urgent Incidental Findings

C: End of study Individual Results
- Study Group Assignment
- 1° Endpoints
- 2° Endpoints
- Safety endpoints

D: Exploratory Results
- Includes exploratory endpoints
- During or after close of study
- May lead to future research

E: Aggregate Results
- 1° Endpoint
- 2° Endpoints
- Summary of Conclusions

Data types recommended for return, at a minimum, are highlighted in yellow

*Accessed on March 15, 2019 at https://mrctcenter.org/resources/?project=return-of-individual-results
Working with PDAI

A collaboration of research sponsors dedicated to the return of individual research results

: To enable trial participants to access their data in a responsible manner that is standardized across pharmaceutical companies.
PDAI - Working to solve the follow-up challenges facing patients, sites and investigators

Patients surfaced the following pain points regarding post-trial follow-up:

- A desire for trial results communicated promptly upon completion of the trial
  - Personal
  - Aggregate
- A desire to know which trial arm (experimental vs. standard of care/placebo)
- A greater sense of closure and appreciation from trial team reflected in clinical trial follow up

Primary Investigators & Nurse Coordinators

Investigators and coordinators mirror patients in the patient request for trial results, however, the following barriers arise:

- Primary investigators and nurse coordinators often do not know trial results themselves until they are published
- Trial results, between writing and peer review, are published a significant amount of time after trial completion
- Patients often ask what trial arm they were on, however the study team is often not informed during or after the study

“Trial results are published and released to the public typically over 1 year after trial completion. At this point, most patients have moved on; Oftentimes we do not know or ever find out trial arm of specific patients.”

– Coordinator, US
<table>
<thead>
<tr>
<th>Patients are in control</th>
<th>Responsibly share information</th>
<th>Not just data, information</th>
<th>Singular intention</th>
<th>Seamless sharing</th>
</tr>
</thead>
<tbody>
<tr>
<td>Protect patient privacy and autonomy by ensuring patients only receive the data they wish to</td>
<td>Return data in a timely manner and withhold only the information needed to maintain trial integrity and comply with regulations</td>
<td>Provide context so patients can understand their data</td>
<td>Create a consistently positive patient experience that remains adaptable to each sponsor’s unique context</td>
<td>Minimize burden on sites, investigators and patients</td>
</tr>
</tbody>
</table>
Innovative Medicines Initiative (IMI) Health Outcomes Observatories

Benefits to Patients:

New platform to empower patients to contribute their outcomes data in a standardized way via digital tools to create transparency of health outcomes for Patients, HTAs and HCPs.

- Collect standardized Patient Generated Data and PROs
- Value-Based Healthcare
- Improved Patient Care & Outcomes

Project Partners:
- Takeda
- abbvie
- Novartis
- Pfizer
- Sanofi
- Lilly
Future focus: Evolving landscape toward Individual Return of Results

- Need for regulatory harmonization
- Address conflict of laws
- Consistency in IRB approach
- Change organizational culture internally & externally
- Develop vendor capabilities
- Further understand patient perspectives
- Conduct pilot studies
- Share learnings and best practices