Patient Engagement Open Forum:

Delivering patient engagement beyond aspirations.

Brussels – September 18 & 19 2019

Report Objectives

To provide a quick reading summary of key presentations attended during the patient engagement forum held in Brussels (sept 18-19).

- Metrics for patient engagement across the life cycle.
- Determining fair market value (FMV) for patient insight into life science R&D.
- Best practice case histories.
- A methodology for a patient engagement in early stage R&D and involvement in clinical trials.

Meeting format

- A two-day event composed of plenary and parallel sessions.
- All sessions were patient driven and facilitated.
- Split was approx. 70% patient and 30% Industry.
- Limited involvement of medical devices companies
- Up to four slides from sessions attended are included in report.

Full slide sets are downloadable from:
https://synapse.pfmd.org/events/dda78c4b-7763-4d74-8360-bcf467b7818a/pe-open-forum/resources

Top line themes arising from the meeting

1. Co-creation for all future activities is preferred way of working with Industry.
2. The success of life science company engagement with patient insight will be dependent on building and maintaining trust and reputation amongst different stakeholders.
3. Future metrics will include non-traditional life science criteria.
4. Patient created contracts for future activities.
5. FMV calculator soon to be launched in US.

Interpretation and implications

1. Identifying compliant FMV for Europe will be an ongoing challenge but needs to be addressed (EFPIA).

2. Patient group capacity will become a determining factor in a group's ability to provide meaningful input in R&D.

3. Many groups not ready for R&D engagement. High levels of motivation but R&D input is not their traditional advocacy role – how best to help them manage this transition?

4. Methodologies for identifying and incorporating patient input into R&D often dependent upon internal company culture, structure and resource availability.

Interpretation and implications

5. R&D-specific compliance guidelines need to be considered (EFPIA).

6. Internal management / coordination of multiple touch points between corporate teams and patient groups / advocates across life will be needed.

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Key presentations

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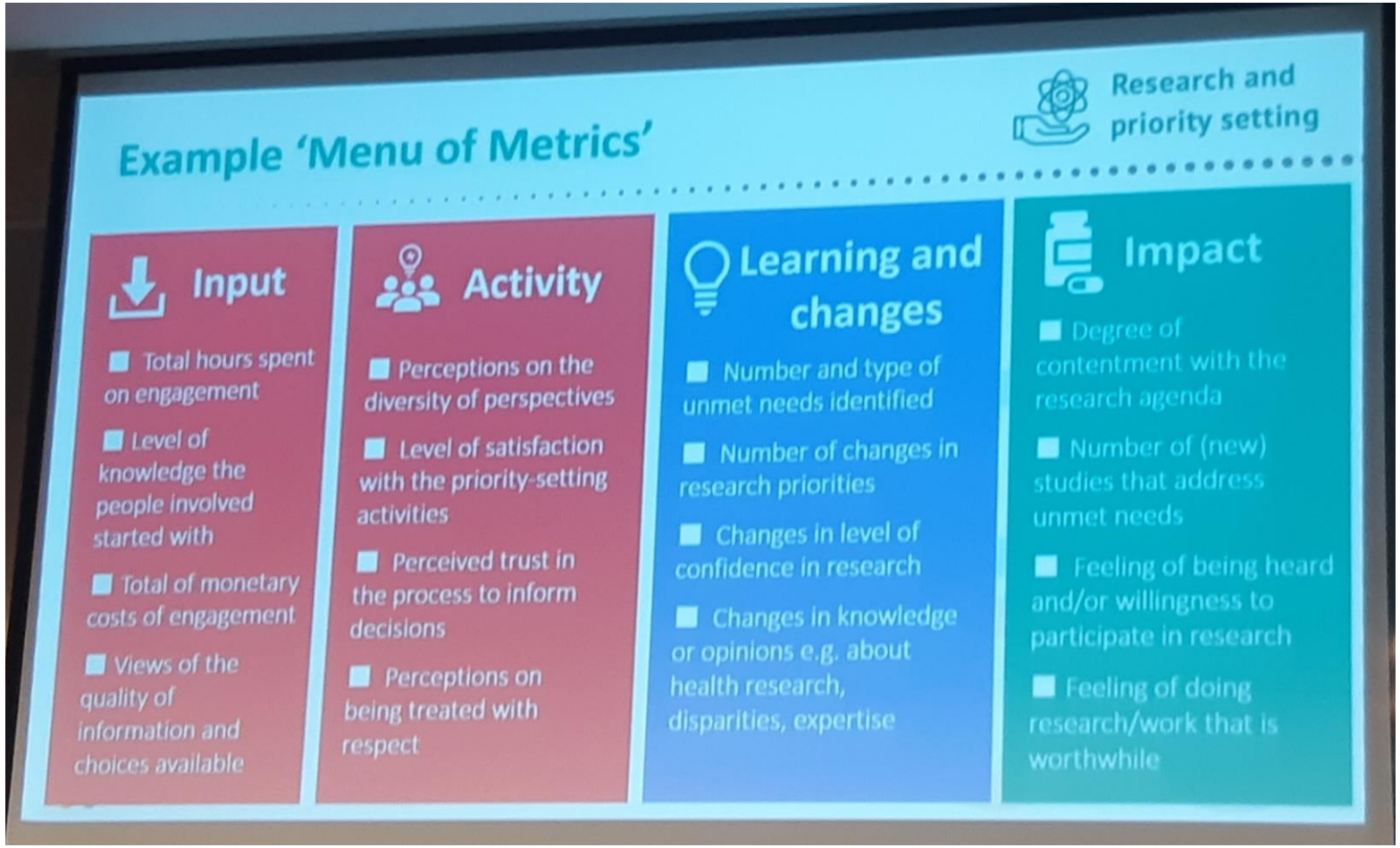
Proposed metrics for patient engagement

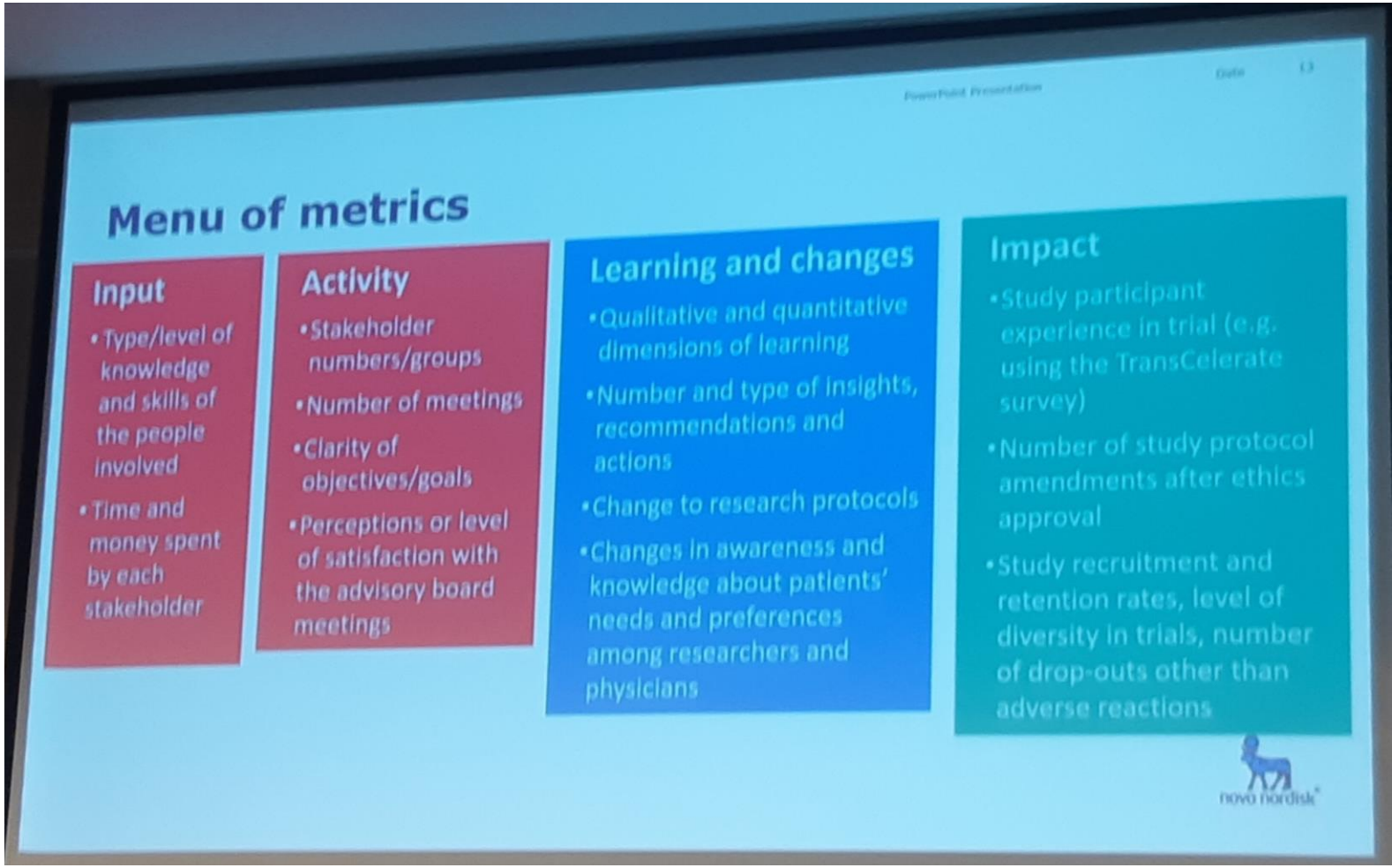
Four metrics:

- Input
- Activity
- Learning and changes
- Impact

Proposed metrics for patient engagement



Proposed metrics for patient engagement



Implications

1. Medical devices and treatments will have different metrics but there will be overlap.

2. Understanding the most appropriate way to identify patient insight in each of these four areas will need to be clearly identified upfront but kept as simple as possible.

3. Low awareness amongst patient community on the influence of compliance of this process.

4. Metrics likely to be adapted on a company to company basis.

Creation of Fair Market Value (FMV) calculator

- Within the next few months the US National Health Council will be launching a fair market value calculator for patient engagement activities.
- The calculator is based on a multi-point validation criteria which covers type of disease, relationship to disease, proposed activity and duration.
- A special category criteria called Modifiers will be applied to each engagement exercise.
- The US version will be adapted for European use via EFPIA.

Creation of Fair Market Value (FMV) calculator

The calculator will be used in three areas:

1. Compensating patients and patient groups
2. Contract templates
3. Conflict-of-interest and privacy principles

Fair Market Value Calculator - What's on the horizon

PFMD Board Meeting May 2019

PATIENT FOCUSED
MEDICINES DEVELOPMENT

Steering Committee

Strong Steering Committee participation from:

Review Committee

Data Collection

"Modifiers"

Activities	Activity Mode	Type of Patient	Amount of time	Additional Burdens	Other modifiers
Interview	Telephone	Patient (with condition)	Hours	Childcare needed	Local travel (<1 hour)
Focus Group participant	In-person	Patient (without condition)	Days	Transportation needed	Short distance travel 1-2 hours
Consultant	Paper	Online forum patient		Caregiver support needed	Very long distance travel (>6 hours)
Presentation (Testimonial)	Verbal	Co-Investigator			Risk (e.g., adboard for DSMB)
Panel Participant	Online	Patient group			Audience size (small group)
Presentation (Keynote)		Patient expert/representative			Audience size (large group)
Survey completion (already created)					Audience size (conference)
Survey of membership					Inconvenience (high)
Reviewer					Inconvenience (medium)
Advisory Board participant (ad hoc)					

Implications

1. Potential competition between patient advocates / patient groups in certain circumstances.

2. Possible disparity between companies who adopt FMV calculator and those that do not may impact patient group management.

3. Companies paying trialists may become a possibility.

Patient Engagement open forum

Beyond Aspiration

Legal Agreements between Patients, Patient Advocates and Pharma

Patient Engagement Open Forum

19.9.2019

PATIENT FOCUSED MEDICINES DEVELOPMENT

WECAN Workgroup of European Cancer Patient Advocacy Networks

MPE Myeloma Patients Europe

Patient friendly contracts

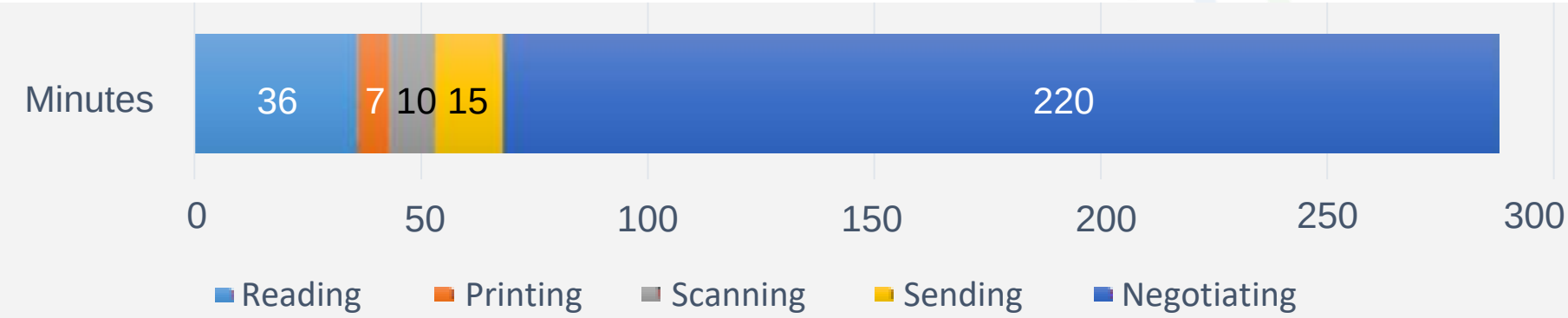
Guiding principles document published which covers scope and examples of contracts.

Contracts developed in response to earlier patient concerns re existing Industry documentation.

Main issues identified in major survey to over 80 patient advocates in 2016

- The contracts provided to patient advocates are often too long and are difficult to understand
- 81% said all contracts are unreasonably extensive in length (6 pages or more, 19% even said they usually get contracts with more than 10 pages)
- Patient advocates invest on average 295 minutes (almost 5 hours) into reading negotiating and processing each contract

How much time (in minutes) do you usually invest for each agreement?



Source: WECAN survey "Reasonable agreements between patient advocacy and the pharmaceutical industry", WECAN (2016)

Guiding Principles document finalized on ratified by WECAN and PFMD

All sections have 3 parts:

1. Rationale
2. Examples
3. Guiding principles

Main issues identified in major survey to over 80 patient advocates in 2016

- Litigation will ruin the organization or individual if ever executed
- Losing the rights on your own ideas and contributions
- Time invested in work not fairly reflected
- Confidentiality of non-sensitive work blocks important patient advocacy work
- Unfair travel conditions for busy patient advocates and for frail individuals
- Unlimited use of photos, quotes and recordings put credibility at risk

Source: WECAN survey "Reasonable agreements between patient advocacy and the pharmaceutical industry", WECAN (2016)

WORKSHOP 1

How to engage patients in early development and preclinical phases of medicines development

Patient Engagement Open Forum
19.9.2019

PATIENT FOCUSED MEDICINES DEVELOPMENT

Patient Engagement open forum Beyond Aspiration

WORKSHOP 2

How to engage patients in clinical trial phases

Patient Engagement Open Forum
19.9.2019

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Beyond Aspiration

Patient insight into clinical trial design and early development

- Both of these initiatives are still work in progress and the final versions are expected end of 2019.
- The early stage involvement appears more advanced as there is a clear framework within which to work.
- The early development approach resembles the classic advocacy planning cycle. Interventions though can be started at different stages rather following the traditional mapping stage.

Patient insight into clinical trial design and early development

- Important questions are; suitability of patient groups / advocates to approach and identification of appropriate patient profiles (profile vs task)

- Involvement and buy in of industry research leaders needs to be addressed as this group has not been involved to date.

Preliminary model to engage patients at the discovery and early development stages

1.Disease/medical condition profile: **gathering patient** insights to understand symptoms/ manifestations

2. Therapy area profile: **understanding from patients** and healthcare professionals how well existing treatment options meet patient needs

3. GAP analysis: **working with patients** to identify unmet needs and prioritise areas of research

Case studies:

1)Anxiety in patients with Parkinson's;

2)HIV treatment solutions (ART)

4. Research methodology:

working with patients to evaluate the optimal tools/approaches to address the research objectives: both lab and clinical (both interventional and non-interventional, including PCOs/PROs)

5. Outcome:

Target Product/Value Profile (TPP/TVP)

Co-designed with patients, using previously gathered insights, to ensure that the TPP/TVP reflects the expected minimally acceptable product's characteristics and desired value to be delivered to patients

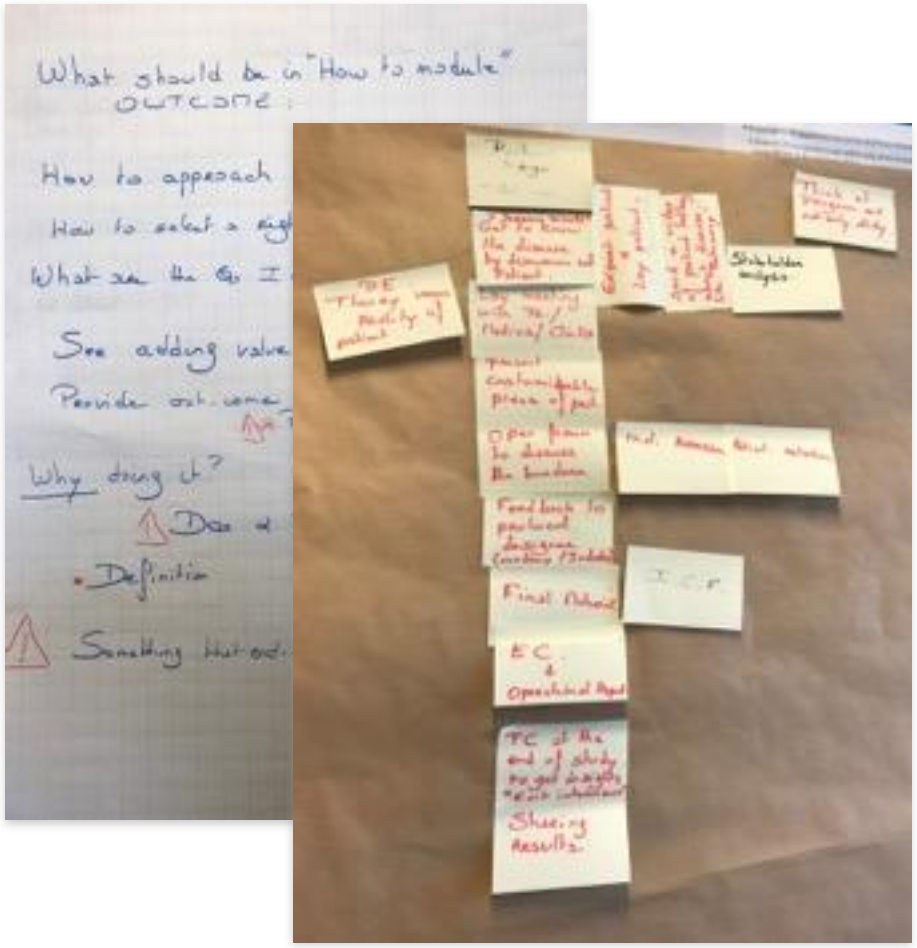
PATIENT FOCUSED MEDICINES DEVELOPMENT

PFMD Working Groups 2019 - Work in Progress

Examples of patient engagement activities and considerations

Stage	Example PE activities
1. Disease/medical condition profile	- Survey to large group of patients - Interviews with patients (questions co-designed with patient steering group)
2. Therapy area profile	- Workshop to bring together healthcare professionals and patients
3. GAP analysis	- Survey - Workshop - Advisory group
4. Research methodology	- Workshop/series of meetings
5. Target product/value profile (TVP/TPP)	- Steering group

Outcomes of team workshop in June 2019



Considerations on what should be included in a "how-to" module for Patient engagement in the protocol design.

- *How to select and approach the "right" patients?*
- *What needs to be considered in the engagement activity?*
- *What's in it for the team and patients involved?*

Draft content expected to be finalised by end of year 2019

Preliminary topics to be included in the how-to module

Key points to consider while engaging patients in protocol set up

- Getting to know the protocol or the program
- Involvement of “expert and lay” patients
- Disease knowledge i.e. Patients’ & caregivers’ insights on the reality of patients’ life
- How to ensure that results are shared with patients

Preliminary topics to be included in the how-to module

Activities where patients can be engaged and contribute

- Study design & Target population (Inc./ excl criteria, medical history,...)
- Study objectives and endpoints definitions to support statistical analysis & innovative approach to support patients unmet needs.
- Patients burdens & study assessments

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