

Metrics to determine the shared value of patient engagement

WP3 workshop Open Forum, September 19th 2019

Welcome

Thank you for being here today!

Meeting objectives:

- To show how to monitor and evaluate PE activities; what metrics?
- To show participants practical examples of evaluating value of patient engagement.
- To discuss the feasibility, strengths and weaknesses of the approaches.

Mentimeter as tool for discussion

Which metrics does the audience think are feasible and meaningful to measure?

Use your phone or laptop to go to:

www.menti.com

Enter the code as shown on the screen

Select the stakeholder group that you represent

A coherent Monitoring and Evaluation framework?

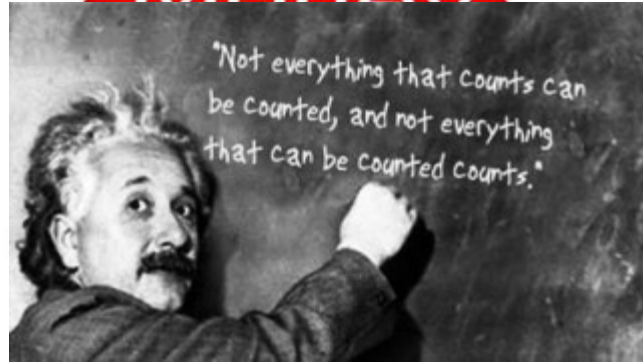
The framework is a tool, a possible answer to the question ‘do we add value, and how’?

- **Problems:**
 - relation input-output is not always clear
 - it might take years before effect becomes clear
- **Solution:** framework connects the dots between inputs, activities, changes and impacts (organisational level/wider context)

A coherent set of metrics?

- Metrics need to be **valuable** for all stakeholders
 - Show relevant value for all
 - Are practical, usable for all
- Focus on **value**, not only on what is easy to **measure**

How to measure actual value?



Not everything that counts can be counted, and not everything that can be counted counts.

(Probably written by) William Bruce Cameron (1963)



What and how to measure the ‘return on engagement’?

Received: 14 March 2019 | Revised: 2 July 2019 | Accepted: 17 July 2019

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REVIEW ARTICLE

WILEY

Evaluating the “return on patient engagement initiatives” in medicines research and development: A literature review

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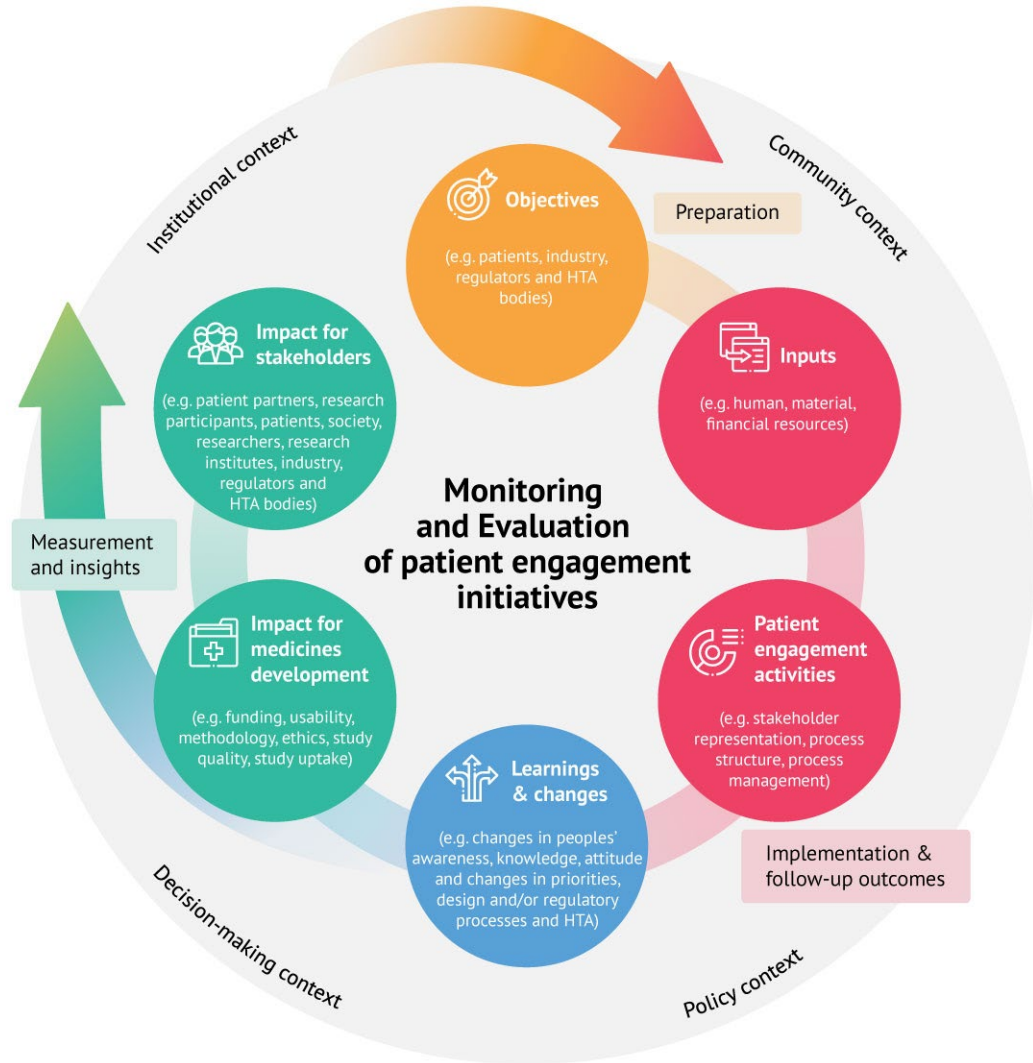


Monitoring and evaluation framework

Includes evidence-based*:

- examples of benefits and investments
- examples of metrics
- examples of factors that may influence success

*Based on literature, case studies and multi-stakeholder workshops





Example 'Menu of Metrics'



Research and
priority setting



Input

- Total hours spent on engagement
- Level of knowledge the people involved started with
- Total of monetary costs of engagement
- Views of the quality of information and choices available



Activity

- Perceptions on the diversity of perspectives
- Level of satisfaction with the priority-setting activities
- Perceived trust in the process to inform decisions
- Perceptions on being treated with respect



Learning and changes

- Number and type of unmet needs identified
- Number of changes in research priorities
- Changes in level of confidence in research
- Changes in knowledge or opinions e.g. about health research, disparities, expertise



Impact

- Degree of contentment with the research agenda
- Number of (new) studies that address unmet needs
- Feeling of being heard and/or willingness to participate in research
- Feeling of doing research/work that is worthwhile

Patient Satisfaction in clinical trials

Novo Nordisk Case Study
By Lukas Eichmann



Situation/initiative

- Industry led initiative, still in the planning phase
- Situation: Clinical Trial participation burden
- Proposed solution: Advisory board meetings to support clinical trial design before and after protocol approval
- Desired outcomes: Reduce clinical trial burden and increase participant satisfaction/experience
- Utilize ad-board format alongside participant experience surveys/interviews during and after trial participation

Framework application

- Team workshop
- Framework as starting point
- Applicability due to timelines?
- Knowledge gap analysis?
- Sequence of framework elements
- Separation of activities between internal (planning) and external (execution)
- Context



Menu of metrics

Input

- Type/level of knowledge and skills of the people involved
- Time and money spent by each stakeholder

Activity

- Stakeholder numbers/groups
- Number of meetings
- Clarity of objectives/goals
- Perceptions or level of satisfaction with the advisory board meetings

Learning and changes

- Qualitative and quantitative dimensions of learning
- Number and type of insights, recommendations and actions
- Change to research protocols
- Changes in awareness and knowledge about patients' needs and preferences among researchers and physicians

Impact

- Study participant experience in trial (e.g. using the TransCelerate survey)
- Number of study protocol amendments after ethics approval
- Study recruitment and retention rates, level of diversity in trials, number of drop-outs other than adverse reactions



EUROPEAN MEDICINES AGENCY
SCIENCE MEDICINES HEALTH

Metrics to determine the shared value of patient engagement – the EMA experience

IMI PARADIGM: Open Forum Meeting 18-19 September, Brussels

Nathalie Bere – Patient engagement
Public Engagement Department - Stakeholders and Communication Division

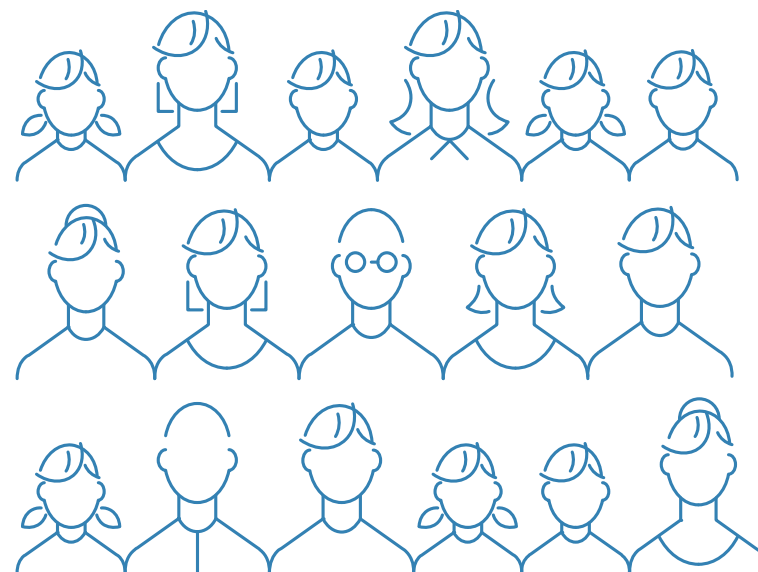
An agency of the European Union



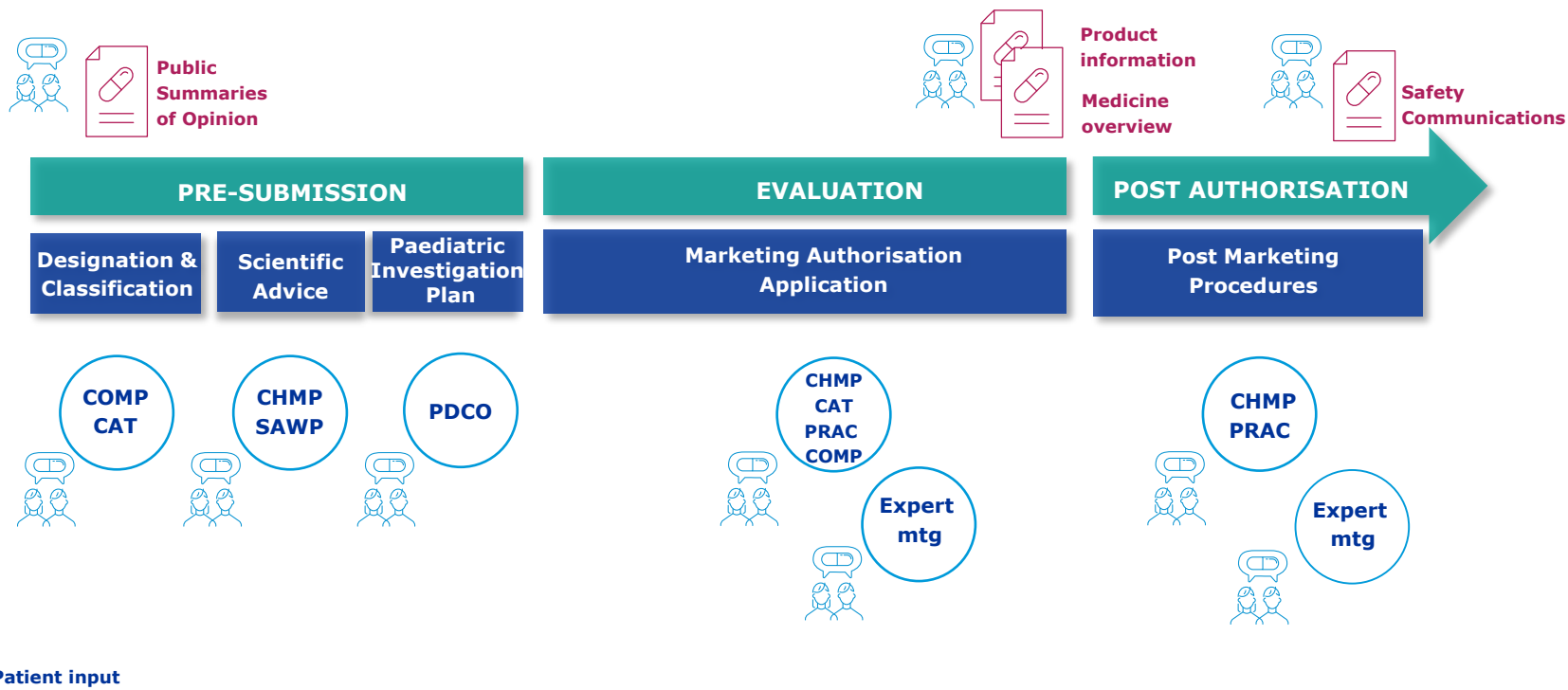


Patient engagement:

- **Integral element of EMA activities**



Patient voice along the medicine lifecycle

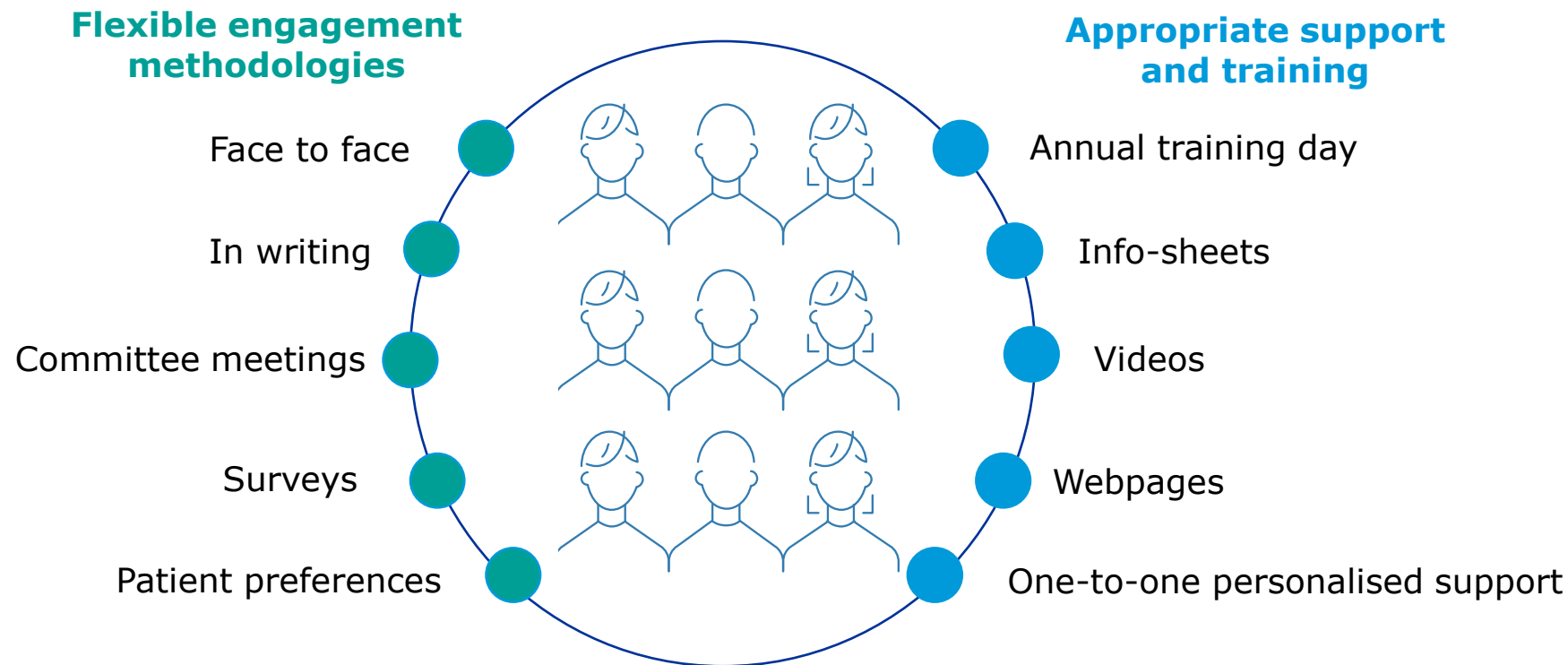




Vital elements



One size does not fit all!



Monitoring and measuring is part of the process



Mutually beneficial?

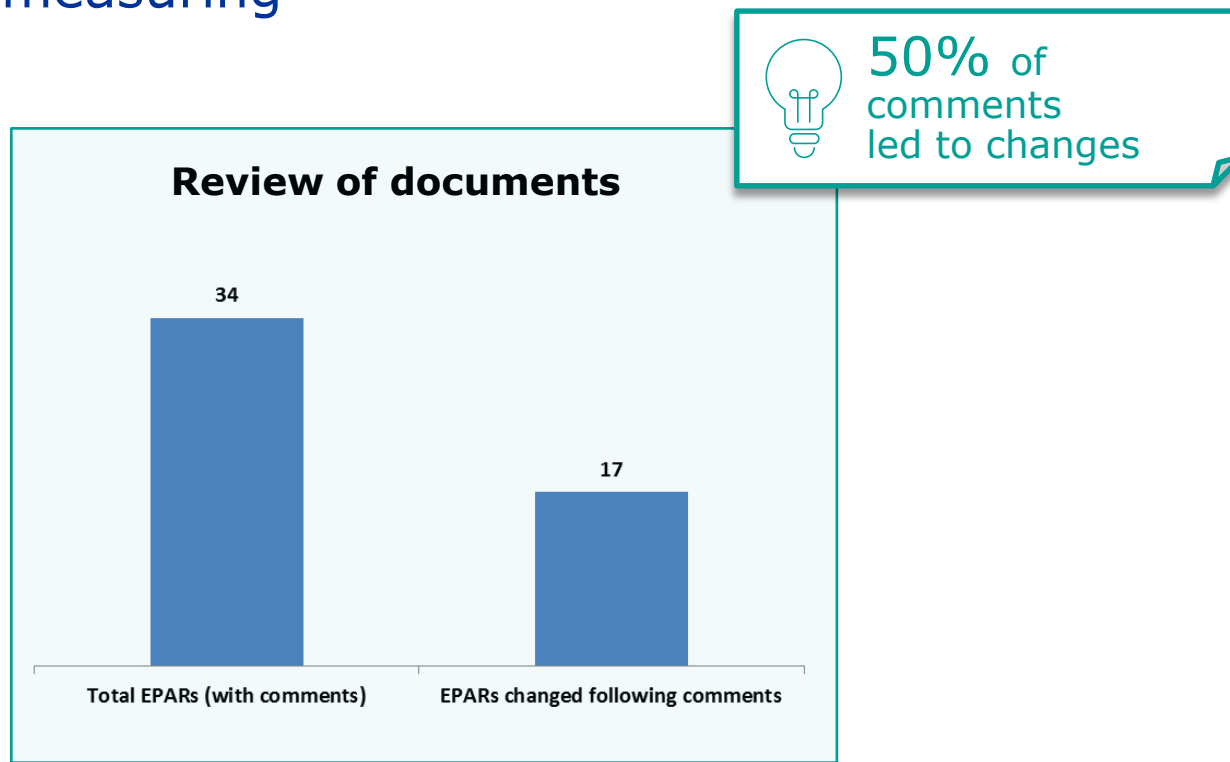


Optimal methodology?



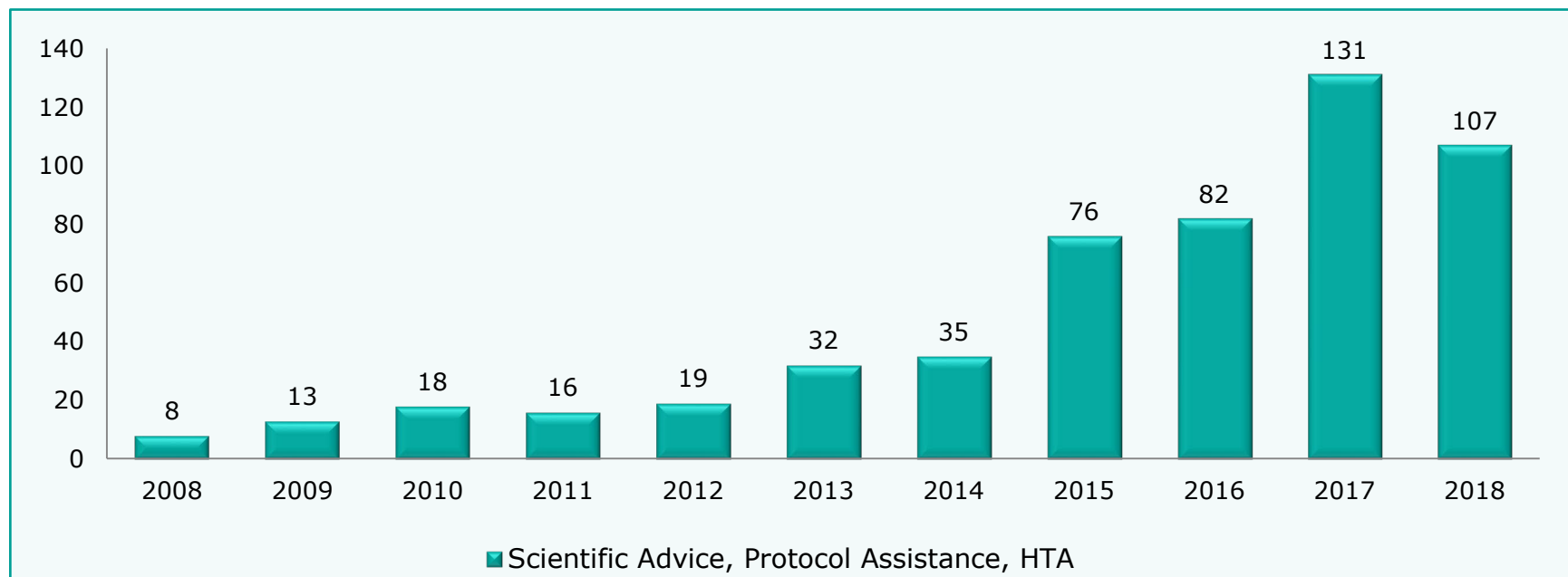


Monitoring and measuring

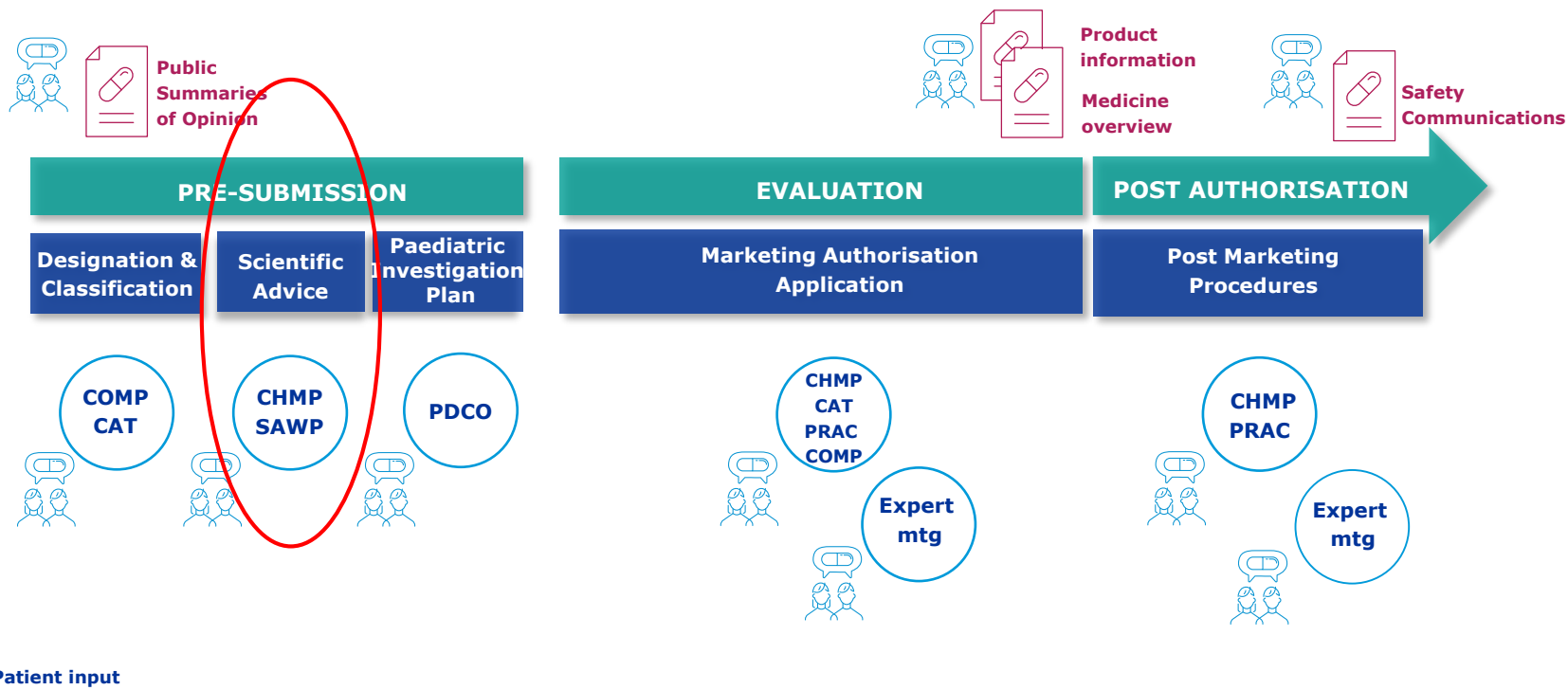




Patient involvement in scientific advice procedures



The patient voice along the medicine lifecycle



Survey for patients

Question type:

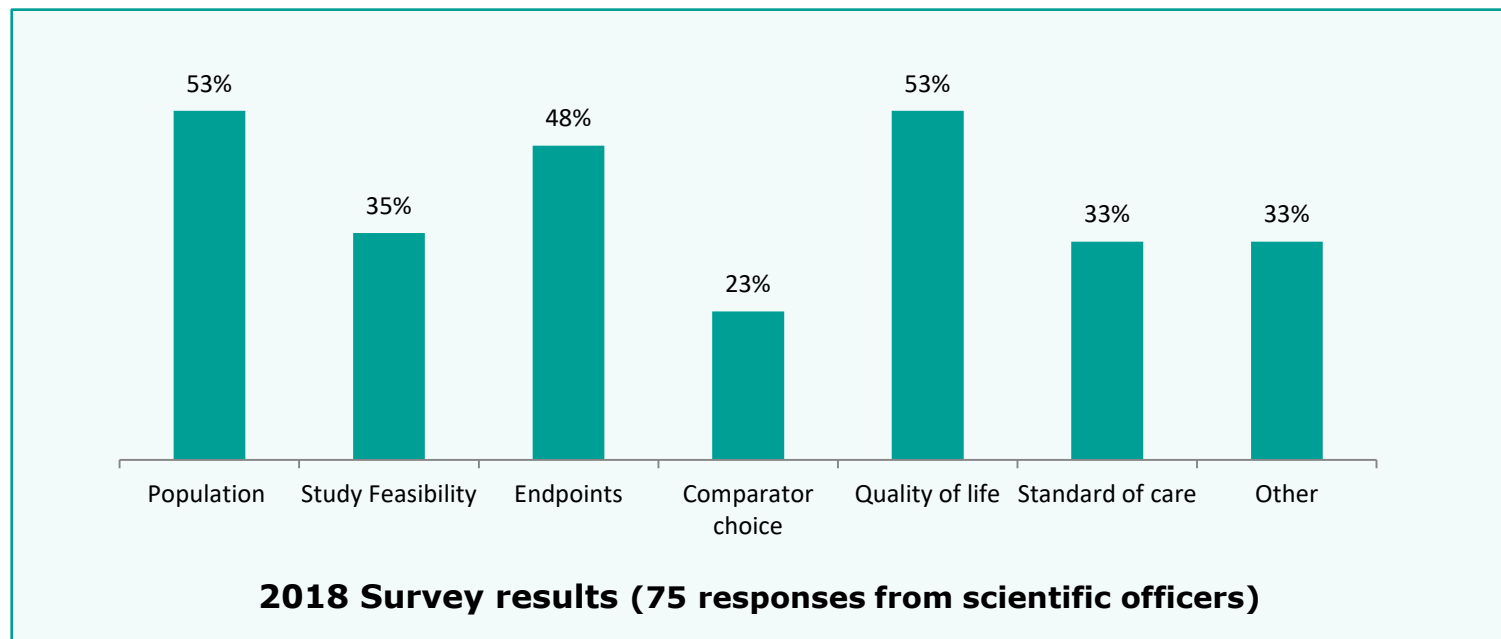
- Were the meeting arrangements well taken care of (travel, access to EMA, etc..)?
- I received sufficient and understandable background information regarding scientific advice/protocol assistance in general / on this specific consultation?
- I understood what was expected of me in terms of my contribution?
- I was able to provide input on the issues under discussion?
- I was given adequate opportunity to provide input to the discussion?
- I feel my comments were taken into account?
- How do you feel overall about your participation in this activity?

Survey for EMA scientific officers

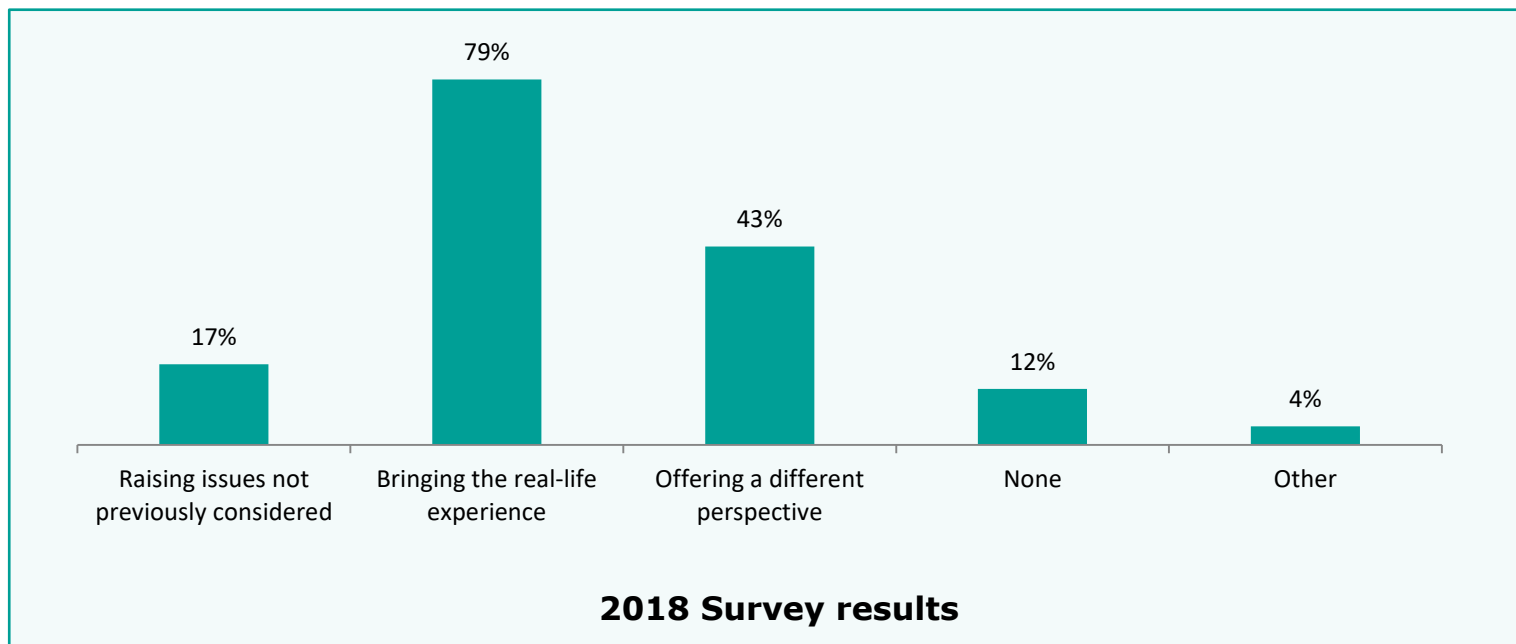
Question type:

- Did the patient seem adequately prepared?
- Did you interact with the patient prior to their involvement?
- In which aspect of the development plan did the patient input?
- Did the patient agree with the proposed responses
- Did the patient`s comments result in further reflection by the coordinators?
- Did the patient`s input result in a modification of the final advice?
- What was the added value of the patient`s input?
- Describe how their input was beneficial

Aspects of development plan where patients gave most input



What was the added value of the patient's input?



Learnings

- Beware of subjective questions!

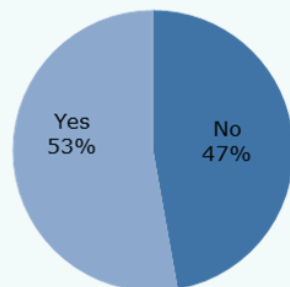
e.g. Definition of impact regarded differently; Patient agreed with plans = impact (yes/no?)

Some considered it impactful, others Not.

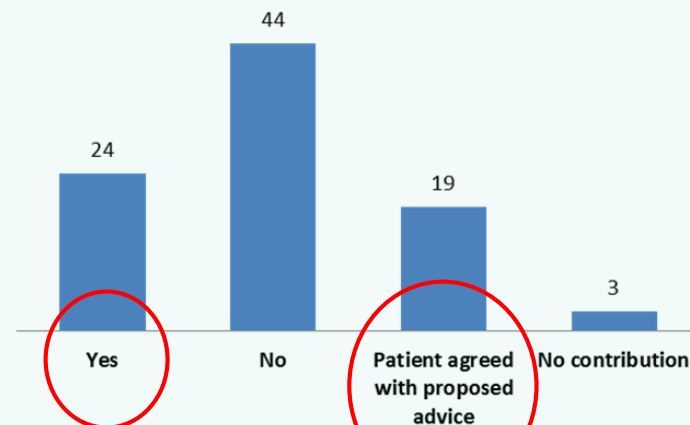
- Refined questionnaire to include more granularity;
 - Before: Did the patient make an impact? / Did it change the outcome?
 - After: Did the patient agree with the proposed responses? / Did the patient`s comments result in further reflection? / Did the patient`s input result in a modification of the final advice? / What was the added value of the patient`s input?
- Wording of questions is vital!

Did it make a difference?

Did the patient's comments lead to further reflection?



Did the patient input result in a modification of the final advice



2018 Survey results



Questions?



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Send a question via our website www.ema.europa.eu/contact



Metrics

Are we succeeding?

How do we know?

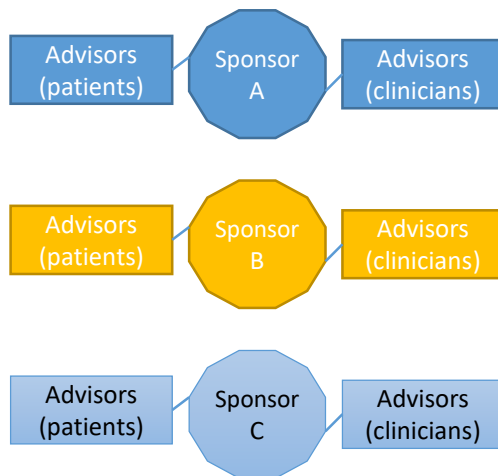
What do we measure?

What do we want to measure?

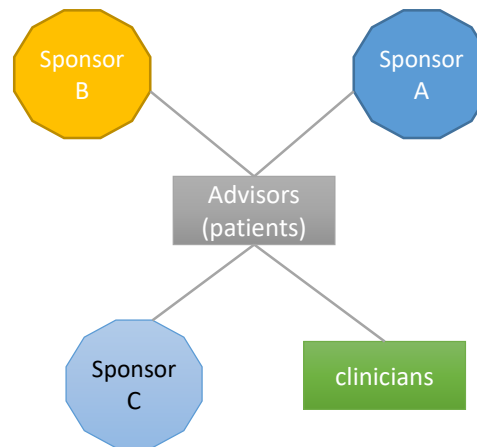
EURODIS.ORG

What are CABs?

Sponsor-dependant model



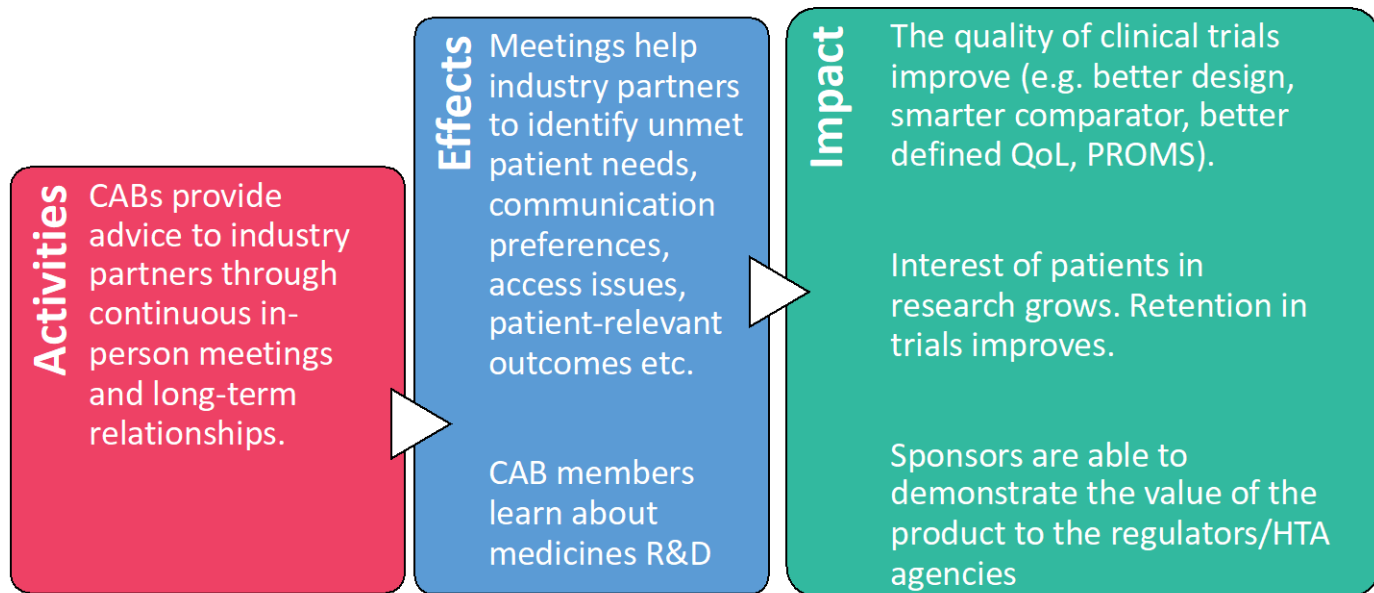
CAB model



Expected outcomes of PE

Less research waste, faster approval and access to drugs and devices.

Mission



Patients with a rare disease have limited treatment options, there is a high unmet need.

Research gap

What to measure?

Input

Type of CAB priorities / goals

Activity

- Perceptions on the usefulness of the meeting
- Satisfaction with the moderation, meeting space, food, logistics
- Number and type of priorities/goals (set before the CAB meeting) discussed during the CAB meeting
- Number and type of requests by CAB members

Learning and changes

- Three most valuable learnings CAB members and sponsors take away from the meetings
- Sponsors' perceptions on if the meeting helped to identify previously unknown or unmet patient needs and patient-relevant outcomes
- Type of response from sponsors (actions/updates) related to the requests of CAB members (e.g. change in outcomes measures)

Impact

- Sponsors' perceptions on if they were able to improve clinical programs
- Sponsors' perceptions on if the meeting helped to demonstrate the value of the product to the regulators/HTA agencies

How do we measure....?

Two evaluation tools:

- 1) “Success tracker” to track CAB priorities, progress on goals, requests and company actions
- 2) Survey to measure learnings, perceived value of the meeting, experiences

Our success tracker (1/3)



For each of our goals, we should track our progress / successes in the below boxes.

Things to track include: where companies said they'd follow up, have they? Where companies said feedback would be taken on board, was it?

Priority area	Goal	Our request	Company action / updates
Clinical Trials & Research (Inequalities)			To be updated
Communication (external, internal, ie PSP)			

Duchenne CAB Evaluation Sponsors May 2019

[SUMMARY](#) → [DESIGN SURVEY](#) → [PREVIEW & SCORE](#) → [COLLECT RESPONSES](#) → [ANALYZE RESULTS](#) → [PRESENT RESULTS](#)

Survey Design

Duchenne CAB Evaluation Sponsors May 2019

Created on 5/21/2019

1 PAGES 14 QUESTIONS

Survey Language: English

Theme: Jungle

Logo added

No logic added

Responses and Status

TOTAL RESPONSES

7

OVERALL SURVEY STATUS

OPEN

NOTIFICATIONS

Edit

ANALYZE RESULTS

Collectors

OPEN

Web Link 1

Created: 5/23/2019

7 RESPONSES COLLECTED

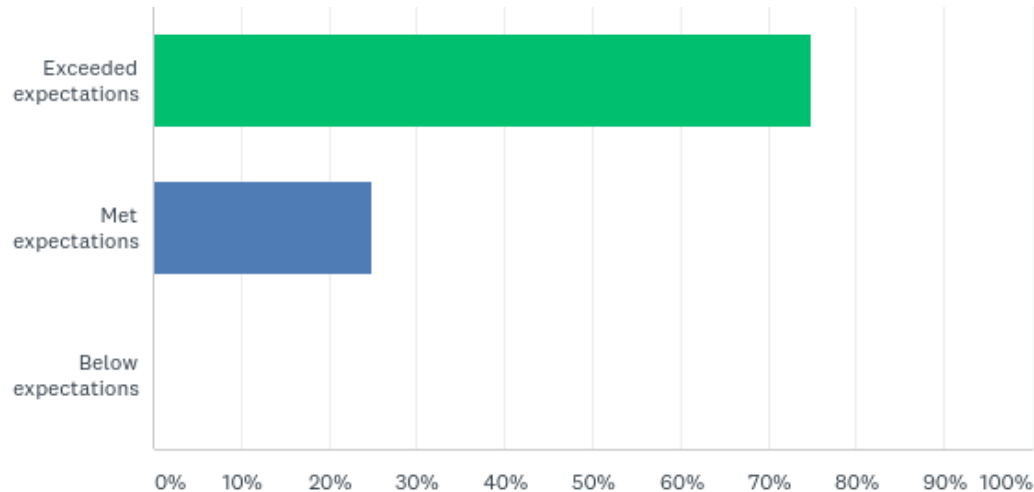
Responses Volume

5

5/13/2019 - 6/1/2019

Example I: survey question and results

Question: How useful was the meeting with the CAB?



Example I: survey question and results

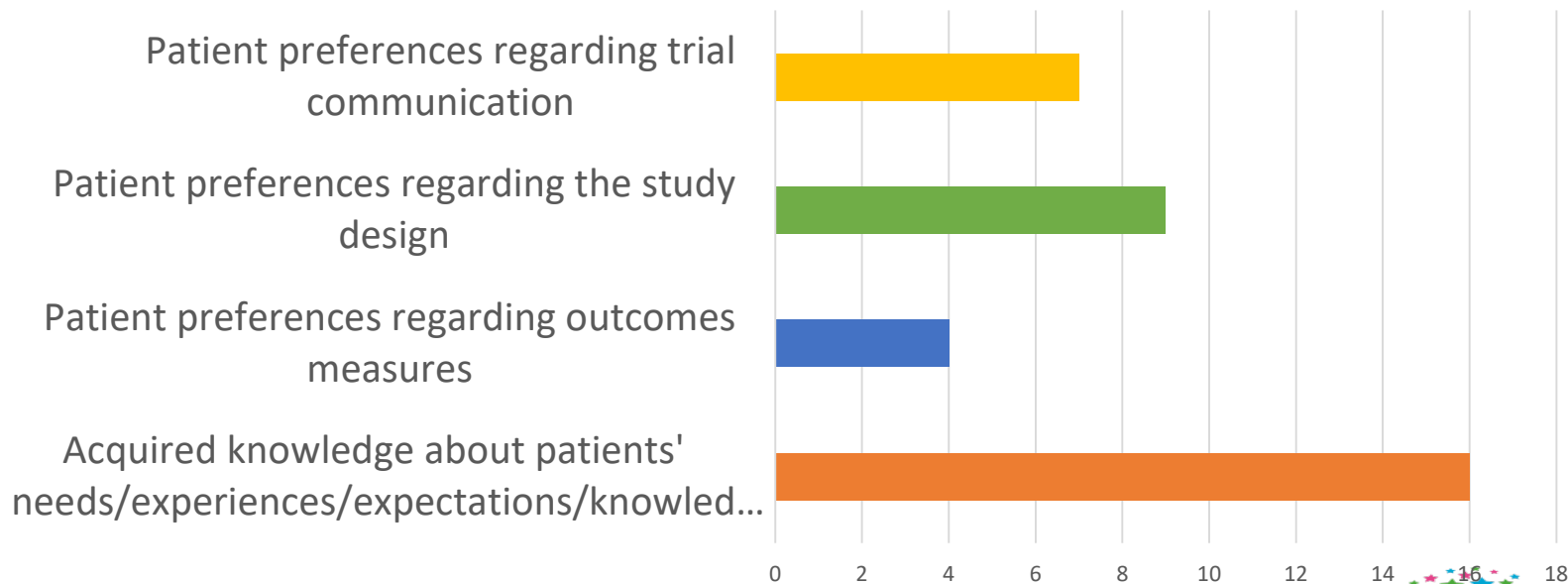
Question: What were the three most valuable things you learned at the event?

“Measurements used in trials are not pairing well with what is important to affected individuals and their loved ones” (sponsor)

“That the outcomes measures taking into consideration do not always reflect substantial changes in patient QoL” (CAB member)

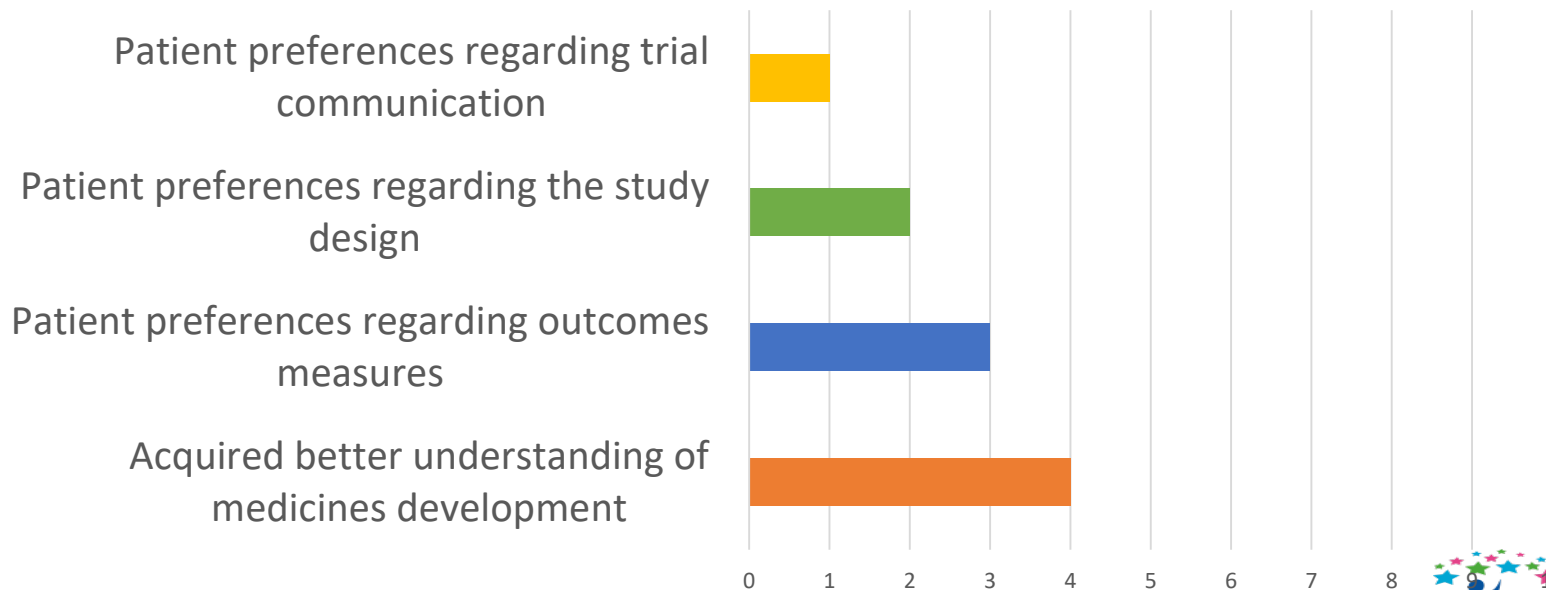
Examples of learnings (sponsors)

Learnings related to clinical trials
(Sponsors, # of quotes)



Examples of learnings (CAB members)

Learnings related to clinical trials
(CAB members, # of quotes)



Example II: Tracker

If overarching goals are refined during discussions with CAB members, this slide will require an update accordingly! N.b. we have included only goals relevant for company meetings, not 'horizontal' goals (e.g. increase # of companies interested)

The below goals apply to all of our CAB meetings. They should be reviewed as a first point of call before all upcoming CABs, prior to setting specific goals for that meeting.

As far as possible, make sure that our specific goals for individual CAB meetings are in-line with these overarching goals.

CF CAB Priorities

Clinical Trials / Research	Closer interaction with companies, facilitating early contact with CT design, to make trials more patient-oriented. Get more companies interested in the CF field, to accelerate research. Increase research in organs affected by CF other than the lungs. Diminish inequalities around Europe.
QoL / PROMs	Work on patient-relevant and patient-friendly measurements of QoL and PROMs.
Access	Inequalities. Improve patient access to new drugs and clinical trials throughout Europe. Inequalities.
Patient Support / Communication	Empower patient representatives through increased knowledge and knowledge-sharing. Inequalities.

CF CAB Goals

Clinical Trials / Research	Increase research in organs affected by CF other than the lungs in X% of CAB meetings by 2020.
QoL / PROMs	Give input about PROM and review informed consents in X% of CAB meetings by 2020. Give input to XXX QoL and PROM measurements before 2020.
Access	Improve access to CFTR modulators in Europe by X% by [date]. Increase the number of new drugs / clinical trials in Eastern Europe by X% by [date].
Patient Support / Communication	Organize X training sessions to CAB members before 2020.

Patients' needs and desires

Access to treatments was central again for this meeting, and we eagerly await an **update on access** issues country by country in Europe. Can there be a database with people on treatment per country by age group, updated in real time?

While opening an office in Poland is hopeful, what are the plans for this office? Will they pledge to get a certain number of “Central” Europeans on XYZ treatments by 2020?

We repeat that communication is key, and perhaps XYZ needs to do more not only with us, but with payers, because there continue to be recurring delays in reimbursements.

We repeat the recommendation that XYZ do more in parsing the patient population to get a better idea of the levels of success on therapies, that do not all have to do with mutation profile.

Compassionate use & exp access – it seems that compassionate use does not exist in most non-approved countries (criteria, application procedure, etc).

Expanding clinical trial sites can be an area of collaboration.

ICFs

Reflections on value

Companies who commit to the CAB process see value quickly

No sponsor has ever expressed disappointment and not come back

There are initial misgivings and concerns from companies who have never done it

We need to measure that value in order to be able to repeat and make the process better over time



THANK YOU.

EURODIS.ORG

Interactive discussion on feasible and meaningful metrics

Use your phone or laptop to go to:

www.menti.com

Enter the code as shown on the screen

Take a patient engagement activity/initiative in mind:

You will select and grade metrics based on whether they are **feasible** and **meaningful**

Feasible: A metric that is easy to measure or collect

Meaningful: A metric that is important to you to measure or collect



Input

- ❑ Type/diversity of perspectives involved
- ❑ Type/level of knowledge the people involved
- ❑ Time and money spent by each stakeholder
- ❑ Type of activity priorities/goals



Activity

- ❑ Stakeholder number/groups
- ❑ Number of meetings
- ❑ Clarity of objectives/goals
- ❑ Perceptions of level of satisfaction with the activity
- ❑ Perceptions on the usefulness of the activity
- ❑ Satisfaction with the moderation, meeting space, food, logistics
- ❑ Number and type of priorities/goals (set before the activity) discussed during the activity



Learning and changes

- ❑ Changes in knowledge about patients' needs and preferences
- ❑ Number/types of recommendations and actions/response
- ❑ Changes to research protocol
- ❑ Changes in knowledge about clinical studies among patients
- ❑ Three most valuable learnings
- ❑ Unknown or unmet patient needs and patient-relevant



Impact

- ❑ Study participant experience in trial
- ❑ Number of study protocol amendments after ethics approval
- ❑ Industry's perceptions on if they were able to improve clinical programs to be more aligned with patients needs
- ❑ Industry's perceptions on if the meeting helped to demonstrate the value of the product to the regulators/HTA agencies

Timeline WP3 2019-2020

- May 23rd Tutorial webinar on how to work with the framework
- June Partners worked with the framework as a tool for evaluation
- July VU-Athena received input and plans a call, provided feedback
- Aug-Sept Calls and workshop at the Open Forum
- Oct-Feb Partners select relevant metrics and start measurement (if possible)
- March/April Ranking of metrics and insights in experiences/barriers
- April/May Alignment workshop (tentative)
- June Final Open Forum, launch/test interactive (online) framework tool
- July-Aug Scientific article on framework, finalize guidance document/tool



Thank you. Stay involved and learn more at: <http://imi-paradigm.eu/>

Contact WP3: T.J.Schuitmaker@vu.nl