ACT: A Community Team

Innovation Impact in Research Award Winner - Celgene, 2015
Uniting Patients with Industry and Researchers

ACT Roundtable
Patient Engagement in Research
Expert Stakeholder Meeting

Hosted by IFAA, February 20th, 2017, via virtual international webinar

IFAA is an International Foundation for Autoimmune & Autoinflammatory diseases with Arthritis as a major component.

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Introduction to A Community Team (ACT)

ACT Project Rationale

Most autoimmune and autoinflammatory diseases with arthritis as a major component are eventually treated with pharmaceutical drugs, like biologics, which cost an average of 2.6 billion dollars to develop and take an average of 10-15 years to get to market. However, since genetic and environmental factors both play a role in triggering these diseases, onset and progression will differ per individual. This, combined with other factors such as delay in detection, atypical presentation, and comorbidities, makes drug development particularly challenging in a varied population, like autoimmunity. Over the past several years, pharmaceutical companies have realized the importance of enlisting patient advisors to understand outcomes, expedite processes, and better utilize research dollars. While enlisting the help of advisory panels is adequate in most areas of research, it is not sufficient in early drug development phases since the treatments must help a diverse group of individuals. Therefore, the same patient advocates (or the “5%” of the population representing all voices), are typically enlisted, which is an inaccurate representation of a realistic patient sample. To advance treatments for those living with these complex diseases, a large group of patients that are representative of all subgroups, even the atypical patient, must be part of the discussion (a.k.a. “at the table”). However, barriers to engagement have existed that limit interaction, including industry regulations and patient inability to participate due to geographic location or disease complications.

ACT Solution

To address these issues, IFAA designed A Community Team (ACT), a planning project that is testing and developing new collaboration strategies to enhance engagement between industry, researchers, and a global pool of autoimmune arthritis patients to improve endpoints, expedite processes, and better utilize research dollars in the early drug development phase (pre-clinical trial) of the rheumatology Research & Development (R&D) continuum. The project is divided into three phases: The Hub, ACT Live, and the Roundtable.

- **Phase One: The Hub (completed August 2016).** The primary goal was to identify known industry and patient barriers to engagement then use that information to develop and design a platform (the “Hub”) that would unite industry representatives with a realistic sample of patients through discussions led by IFAA and patient advocates. Once developed, the second goal was to test research methods, including 1) if IFAA could bridge communication between industry and a large number of patients and 2) if research led by patients with experts as advisors could produce robust data. While the research was conducted to test and develop procedures, results led to further discussions that concluded most patients ideally hope to combine both pharmacologic and non-pharmacologic therapeutic options to address their complex disease needs. For this reason, IFAA has begun studying barriers and benefits associated with all current methods of patient engagement so that future collaboration strategies between pharmaceutical and non-pharmaceutical researchers can be developed.

- **Phase Two: ACT Live (completed November 2016).** The primary goals of the second phase of ACT were to further review barriers and benefits associated with patient-research engagement, per stakeholder group and within subgroups. To test new methods that reach patients who are not typically “at the table,” patient leaders from the Hub conducted interviews with select non-pharmacologic researchers and patient advocacy
organizations (nonprofits), which were streamed live to Facebook. This provided any patient the opportunity to participate, regardless of geographic location or advocacy experience. Interviews with nonprofits were conducted primarily to learn how they have incorporated patient-centered research into their work. Due to regulatory barriers, industry researchers were not able to participate in the open forum of ACT Live, so separate interviews were conducted with five pharmaceutical companies to explore how their internal barriers and future corporate goals could be considered and personalized into future Hub strategies.

- Phase Three: The Roundtable. This final phase of ACT, hosted as an online, interactive meeting with stakeholders from around the world, all identified as leaders in patient-research engagement, will review the history of collaboration and associated methodologies, discuss known barriers and benefits to collaboration, and determine future best practices as the landscape evolves and new parties enter the space.
Roundtable Report

Information provided in this summary is based primarily on data provided by participants during and following the Roundtable, via panelist interviews, real time polls, and responses to polls and discussion questions.

Panelists

Moderator: Tiffany Westrich-Robertson, ACT Project Manager & Patient-Researcher Liaison, CEO of IFAA. Patient living with Rheumatoid Arthritis, Axial Spondyloarthropathy, Sjögren's Syndrome.

Panelists

Cheryl Koehn, Founder and President, Arthritis Consumer Experts and the JointHealth® family of programs. Rheumatoid Arthritis patient.

Maarten de Wit, OMERACT, PARE, VU Medical Centre Amsterdam, Lead author of published definition of Patient Research Partners (EULAR). Psoriatic Arthritis patient.

Kaleb Michaud, Co-Director, National Data Bank for Rheumatic Diseases (NDB), Assistant Professor, Rheumatology & Immunology, University of Nebraska Medical Center (UNMC). Rheumatoid Arthritis/Juvenile Idiopathic Arthritis patient.

Rebecca Schumacher, Executive Director of Arthritis Research Center Foundation and National Data Bank for Rheumatic Diseases (NDB)

Invited Participants

Pharmaceutical Industry, Nonprofits, Patients, Non-Pharmacologic Researchers, Corporate Research Organizations (CROs). Attendees were told prior to the webinar that the meeting would not be an overview of Patient Engagement practices, as those invited were expected to be ‘experts’ on the topic. Those unable to attend in real time were given the opportunity to view a recording of the Roundtable and answer all Polls and Discussion Questions posed during the session.

*Pharmaceutical Industry representatives invited to attend the Roundtable were those who, in addition to being identified as patient-centric, successfully completed a pre-session interview with IFAA regarding perceived barriers and benefits to patient engagement in the research process.
Methods

The Roundtable incorporated Polls & Discussion Questions throughout the session. Attendees, which had representation from each stakeholder group, were polled in real time and answers to Discussion Questions could be submitted during or after the session.

A major component of the ACT project was to find ways to build on current methods of engagement—including broadening the patient sample and creating flexible participation—by removing geographical boundaries and disability barriers via virtual, online engagement platforms. This same philosophy was used during the Roundtable, which united representatives from all major stakeholders around the world to take part in the same discussion. Those who were not able to attend live, due to prior commitments or time zone issues, were also provided with a link to the recording and a survey that included all Polls and Discussion Questions from the Roundtable. Commenting was open through March 30th; any post-session responses are included in this summary.

Of those who participated in the Roundtable, 19 contributed to Polls and Discussion Questions (5 Researchers, 4 Patients who are also Researchers, 3 Nonprofit Leaders, 3 Industry, 3 existing Patient-Research Partners, 1 Corporate Research Organization Representative, 1 Patient-Research Partner/Researcher/Corporate Research Organization Representative)

This full review is considered a “living document”, via Google Documents, open for commentary from all invited Roundtable guests. All comments will be reviewed and added to the document, which will be continuously updated with appropriate revision numbers.

Discussion

Figure 1: History of Rheumatology Patient-Research Engagement DRAFT
To review the history of patient engagement in rheumatology, Tiffany Westrich-Robertson interviewed Cheryl Koehn, Maarten de Witt, and Kaleb Michaud, all who have unique experiences and contributions to its’ evolution. Based on these discussions, in conjunction with information submitted by attendees, a timeline (Figure 1) and summary was drafted.

**History of Patient Engagement in Rheumatology**

Patient engagement in rheumatology research started in the late 1990’s in Canada and around 2000 in Europe\(^1\). Partly due to regulatory barriers between patients and pharmaceutical companies communicating prior to 2009, early patient engagement focused strongly on non-pharmacologic research practices. But, the pharmaceutical industry has now adopted the practice, and calls to push engagement to new levels are driving efforts to build on these established practices of engagement. As innovative collaboration models develop, and the pool of participants multiplies, it will become essential that all stakeholders understand, and respect, existing engagement methods and be able to identify where their work falls in the engagement continuum. Additionally, new efforts must be tracked in some manner, otherwise we may branch off in so many directions that we fail to measure the value of patient engagement and risk duplicating efforts, thus wasting research dollars.

**Non-Pharmacologic Research**

During the early years of patient engagement in rheumatology, various methods of patient-researcher collaborations emerged. Some were implemented by individual groups - such as at Arthritis Research Canada - while others designed official protocols (such as Patient -Research Partners/PRPs\(^2\) which was defined by EULAR in 2010, but initially implemented by OMERACT in 2002). PRP was later adopted by working groups such as GRAPPA and IDEOM to help improve outcome measurements. A key methodology in PRP is uniting patients and researchers as equal partners in the project.

In 2011, the United States established PCORI, and with it defined “Patient Centered Research Outcomes” (PCOR). PCORI enlists a patient per grant review team, ensuring dollars spent are aligned with research outcomes that are meaningful to the patient population. For both PRPs and PCOR, patients are provided some level of research education prior to participation. **While both methods of PRP and PCOR involve patients in the research process, the way patients participate varies slightly (See Figure 2). This becomes particularly important as we advance patient engagement efforts and split from existing protocols.**

Both PRP and PCOR projects have demonstrated measurable value from incorporating patients on projects. PCOR, which began primarily with patients reviewing grants, has expanded exponentially, from consideration in trial design to the development of instruments used in those trials. PRP procedures have been used to establish guidelines in Rheumatoid Arthritis clinical studies\(^3\) (OMERACT, 2015) and currently to revise core domain sets for Psoriatic Arthritis

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1. While patient engagement in research originated much earlier in cancer and the AIDS population, and what has been learned from those efforts should not be ignored, the needs of rheumatology patient outcomes is quite different than those living with more immediate life-threatening diseases, and therefore the purpose of the Roundtable was to focus specifically on work done within the rheumatology community.

2. EULAR’s Patient Research Partnership defined publication, 2010: [http://ard.bmj.com/content/annrheumdis/70/5/722.full.pdf](http://ard.bmj.com/content/annrheumdis/70/5/722.full.pdf)

This work may even become key for bridging non-pharmaceutical and drug development research, as these efforts are relevant to all types of clinical trial design.

Pharmaceutical/Drug Development Research

In approximately 2009 pharmaceutical companies began realizing the benefits of patient involvement in research, but legal barriers minimized engagement. By 2015 most had formed ‘patient engagement’ departments and regularly enlisted the help of Patient Advisory Panels in all phases of the R & D continuum, which has now become an industry standard.

Since efforts in patient engagement prior to 2010 were mostly focused on non-pharmacologic research, which used methods that did not require inclusion of a large patient sample in their design, these collaborative models are problematic for patient engagement in the drug development environment. This need for change has spawned calls for expanded PCOR efforts, often with the help of nonprofit organizations and/or registries, as well as innovation challenges (like the ACT project). However, as innovative methods develop, it will be important to gauge how they fit in with - or differ from - existing, defined methods of engagement so efforts are not duplicated and the value from having patients involved in the research can be appropriately measured.

Involving patients in the regulatory process also started in the 1980’s, as a response to the HIV/AIDS and cancer communities work in patient engagement, with the Patient Representative Program at the U.S. Food and Drug Administration (FDA) and in 1995 at the European Medicines Agency (EMA)\(^5\). Efforts to further patient engagement at both agencies have expanded, including a major initiative at the FDA in 2012 – Patient Focused Drug Development (PFDD)\(^6\). The PFDD heavily relies on PCOR to provide information about what outcomes are most important for patients. Many current initiatives, including ACT, aim to collect enough robust data from the patient community to contribute to regulatory sessions, showing how patient engagement efforts, in addition to methods, are already overlapping.

Databanks and Registries

Databanks and registries are another means to collect relevant data from patients that can be used in both non-pharmaceutical and drug development research efforts. Groups such as the National Data Bank for Rheumatic Diseases (NDB)\(^7\), a patient-reported research data bank that aims to advance knowledge about the causes, outcomes, costs,

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\(^7\) National Data Bank for Rheumatic Diseases (NDB) [https://www.arthritis-research.org/](https://www.arthritis-research.org/)
treatments, and results of treatments related to rheumatic conditions, push patient-research engagement even further. Other advances in PCOR have led to projects like ArthritisPower by CreakyJoints, the first patient-led, patient-generated, patient-centered research registry for arthritis, bone, and inflammatory skin conditions, developed as part of PCORnet, the National Patient-Centered Clinical Research Network, a large, national network for conducting clinical outcomes research⁸.

**Conferences**
Patient engagement in research is increasingly prevalent, including at conferences. Since the launch and success of “Patients as Partners” (conferences hosted in both the US and Europe since 2014), additional conferences that focus on innovation and patient engagement integration are on the rise. However, while “value” is a primary focus, the history of engagement and explanation of existing methods are not explained or defined. This becomes a problem as those new to these conferences – including any nonprofit organizations, researchers, and patients who are attending to learn more about how to engage - may not be familiar with established methods. This was demonstrated in the Patients as Partners US conference in March of 2017, as several attendees expressed confusion regarding terminology (specifically that “PRP” did not make sense and should be redefined), not realizing that the term is not usually transferrable to engagement between industry and patient stakeholders. As efforts continue to grow, particularly as new engagement methods are introduced and defined, it will become necessary to ensure some level of education about the history of patient engagement is available at these events.

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⁸ ArthritisPower, CreakyJoints: [https://creakyjoints.org/research/about-arthritispower/](https://creakyjoints.org/research/about-arthritispower/)
Expanding Current Methods of Patient Engagement

As patient engagement reaches new levels, we need to preserve its history while embracing new, innovative ideas. But where do these new projects fit into the existing continuum? In ACT, elements of both PCOR and PRP were utilized, however, parts of ACT do not fit within either of their definitions. For example, ACT flips the leadership roles of typical PRP engagement, where the researcher is usually the lead and the patient is usually the advisor. In ACT, the patients were trained to be project leaders and the researchers contributed in an advisory capacity. Also, topics explored in qualitative discussions (part of the Agenda Setting phase of research) were mostly based on what IFAA learned is important to patients, rather than based on questions developed by researchers or industry. So when a project does not meet the existing criteria for defined methods, what needs to happen? We posed this question to Roundtable attendees:

**Discussion Question #1**

Given patient engagement is expanding, and will continue to evolve as new, innovative methods emerge, should we consider defining another level of patient engagement (“Patient Initiated/Involved Research (PIR)”) or do we expand on the existing definitions?

**Discussion Question #1 Conclusion**

- The majority of attendees stated that as patient-researcher engagement expands, particularly if it distinctly differs from any official definition published, then a new definition would be necessary to formalize activity and methods.
- If, however, there are only minor adjustments to the methodology, and the process does not compromise the integrity of the existing method, the new process should be considered a ‘variation’ of an existing method.
Additionally, as innovative ideas continue to evolve, best practice would be to expand on existing definitions whenever possible, and develop new definitions only when the methods have been fully developed and tested, are clear, and can be recorded in a way that can be reproduced and measurable.

Regardless if the definitions are expanded or newly defined, delineating any differences in methodology based on pharmacologic and non-pharmacologic collaborations will be beneficial for future value measurements.

In addition to determining what adjustments to the methodology exist, since patient involvement/leadership can range from agenda setting to post-market research, the phase of the R & D process (which is different in pharmaceutical and non-pharmaceutical design) should be noted.

**Summary.** Based on recommendations outlined above, a project like ACT, which combines existing methods, should label itself as a *mixed methods project* and cite which methods are included (in this case PRP and PCOR). Any new methods being tested should be labeled as such and should include the phases of the R & D project. Once testing is completed then the project team can determine how much, if any, the methods meet existing definitions. If they only vary slightly, the project team should note the differences but not establish a new definition. If, after testing, it is determined the new method(s) do not meet the defined criteria of existing methods, clear standard operating procedures should be developed and a method designed and published.

Furthermore, several Roundtable attendees felt that when developing future projects, like ACT, that are designed in part to test methods to overcome barriers, understanding which barriers are most important to which group will be a key factor in the design. Additionally, as we move forward with a collaborative approach to combat these barriers, it may be important to understand which stakeholder has most ‘expertise’ with which obstacle so we can let them lead and/or learn from their work. - Ben Nowell, Global Healthy Living Foundation, Kaleb Michaud, National Data Bank for Rheumatic Diseases, Jennifer Horonjeff, PhD.

**ADDED SINCE THE ROUNDTABLE**

**Patient Centered**

**Defined:** Focused on the patient or consumer of healthcare rather than on healthcare providers, financiers, insurers, or institutions.

**Patient Centered Outcomes Research (PCOR)**

**Defined:** PCOR, 2011/2012
- Assesses the benefits and harms and outcomes important to patients
- Inclusive of an individual’s preferences, autonomy, and needs (QOL)
- Incorporates a wide variety of settings and diversity of participants

**Patient-Oriented Research**

**Defined:** Research focused on patients for evidence-informed health care, a relatively recent concept of clinical care that was largely pioneered in Canada
- It encompasses both clinical research and health services research (for public policy), the synthesis, dissemination and integration of this new knowledge into the health care system and into clinical practice.

**Mixed Methods:**

PRP - Patients who are not existing researchers or doctors identify the research problem then they take a leading role in the project (Moderator, Research Question Design, Analysis)- with researchers in the advisory roles, if they are “equal”, then it could be considered PRP.

PCOR - Focus Groups assessed benefits and harms, preferences, and quality of the outcomes important to patients, as well as including diverse group of respondents.

New for testing: Patients identify and form the research questions.

**Patient Research Partners (PRPs)**

**Defined:** EULAR, 2010
“Persons with a relevant disease who operate as active research team members on an equal basis with professional researchers, adding the benefit of their experiential knowledge to any phase of the project.”

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*Figure 4 Updated Patient-Research Engagement Continuum*
Respondents were asked to submit additional patient engagement methods that were not included in Figure 3. Figure 4 is the updated Patient-Research Engagement Continuum based on Canada’s SPOR projects, which defined “Patient-Oriented Research”. Also, based on Discussion Question #1, ACT is updated to be a Mixed Methods project, not currently defined as a separate category.

**Benefits and Barriers to Engagement: Researchers, Industry, Patients**

Data presented in this section were based on a combination of research collected from publications by other groups, interviews with various pharmaceutical companies, qualitative discussions with patients involved in the ACT “Hub”, 22 interviews conducted with non-pharmacologic researchers and nonprofits during ACT LIVE (Phase II), and comments provided by ACT Roundtable participants.

**Benefits**

Over the last several years, the benefits associated with involving patients in the research process have been noted, yet there are still some members of stakeholder groups who are either not convinced of its’ value or who still have too many barriers to be engaged. **But patient engagement is still surging and branching off in new directions, and with this comes the responsibility to ensure its value is measured in meaningful ways.**

**Barriers**

During the Roundtable, we focused strongly on the barriers identified in patient engagement, per the following key stakeholder groups:

- **Industry**
  - Pharmaceutical Company, independent of their research staff
  - Pharmaceutical/Drug Development Researchers, independent of the pharmaceutical company they work for
- **Non-Pharmacologic Researchers**
- **Patients**
- **Nonprofits**

Attendees were asked to participate in live polls to assess which barriers, per stakeholder group, they believed were the biggest obstacles to advancing patient engagement.

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*After IFAA interviewed stakeholders, it became apparent that barriers and benefits to patient engagement reported by “Industry”, or the pharmaceutical company representatives who work in patient engagement departments and usually arrange collaborative efforts, were different than those reported by the pharmaceutical/drug development researchers working in the labs.

**Results from Polls 1 and 2:**

**Non-Pharmacologic Researchers**
In regards to Non-Pharmacologic Researchers, most nonprofit respondents felt a lack of funding would be the biggest barrier, most patient respondents felt disease unpredictability was the largest issue, and most researchers (regardless of type of researcher) believed the biggest issue was an inaccurate sample. **Industry did not respond.**

**Industry (pharmaceutical company, independent of their research staff)**
In a subsequent poll, we asked attendees to choose which barrier they felt would be most cumbersome for Industry (Company) engagement. The results, based on 19 responses:

- Internal bureaucratic processes/regulatory boundaries, including uncertainty how to engage with patients without crossing legal or ethical lines (n=10)
- Concern regarding trust of patients/perceptions of pharma (n=2) – both respondents were patients
- Sometimes difficult to identify the right patients, particularly for global markets (n=3)
- Using only educated patients creates a bias when we are trying to prove measurements (n=4)
- Addressing flexibility needs (flares, disability) so the right patients are “at the table” (not chosen by any of the respondents as the most cumbersome barrier)

**Pharmaceutical/Drug Development Researchers**
During the pre-Roundtable interviews, we were able to speak with Pharmaceutical/Drug Development Researchers from two companies. Their reported barriers to engagement were slightly different than those reported by both their parent company and Non-Pharmacological Researchers. In addition to internal bureaucratic processes/legal boundaries, Pharmaceutical Researchers also reported the following barriers to engagement:

- Since they are not usually present to speak directly to Patient Advisory Panels, the data interpretation may be based on lead to more questions that can When the pharmaceutical companies filter reports from patient panels to their researchers, as opposed to researchers directly observing or engaging in those panels, the researchers may question data interpretation – particularly if the data is based on a small patient sample. Having a way to establish an ongoing dialogue with patients  *(Note: IFAA tested this barrier in ACT Phase I, as a patient was part of the analysis team).*
- Failure of the company to train researchers to understand benefits of patient engagement

We asked Roundtable attendees to weigh in on their thoughts regarding how patients could engage more directly with Pharmaceutical Researchers:
Discussion Question #2

While patients are “at the table” with industry (patient engagement teams) they are not typically interacting with the researchers responsible for developing treatments. How could we move patient engagement forward to be inclusive of ALL researchers, not just non-pharmacologic?

Discussion Question #2 Conclusion

● Patients are interacting with drug development researchers within CROs and also those working for pharma. We regularly have patients interacting with researchers. - Valerie Powell, MAPI Group  (Note: mentioned during Roundtable, using CRO’s in conjunction with nonprofits/patient groups would be a smart strategy to help establish processes that address the barriers associated with legal limitations.)

● Continue building innovative ideas/methods and testing them (like ACT) and log all results, showing added value. This can include conducting research in collaboration with data banks (ACT II), which provides a scientific foundation to support, and potentially combat, internal company bureaucracies. Additionally, patient engagement isn’t “new”, it’s just new to industry. Once we chart what we’ve learned from non-pharmaceutical patient-researcher engagement, and begin to cross reference the barriers and benefits associated with that work to those that may present differently in drug development, we will have a solid base to grow from and prove value. - Tiffany Westrich-Robertson, IFAA

Patients

The realization of the need to identify specific subcategories within stakeholder groups continued as we analyzed responses from Patients. During our research, we did not find any publications where the patient was divided into subcategories, perhaps because until around 2015 only a small percentage of ‘educated’ patients participated in research collaborations? But even within that context, there are levels of patient expertise, as in many cases the patient is also a research professional.

These “patient-researchers”, some who attended the Roundtable, largely report that their own disease etiology and recognizing how their knowledge as a patient could better projects and outcomes led them down this path. In early patient-researcher engagement efforts, many of these patients were the first “at the table”, partly because they could speak the language.

Barriers to engagement reported by patients who have been “at the table” stated:

● Time involvement is a burden (labor intensive to an already busy schedule); some noted with diseases that extra work makes it “too much”

● “Tokenism.” This is when researchers only bring patients on a project because the grant requires them to do so, but once the project starts the researcher involves them very little, if at all.
● Research team does not make an effort to at a level understandable by the patient participants.
● PCOR Grant Review Specific: Some other stakeholder reviewers dismiss patient comments and/or insinuate that non-patient opinions are more important to the review.
● One voice cannot possibly replace 1,000’s, even though we do our best to represent the population (*Tested in ACT Phase I*)

Patients who are also Researchers were not asked about their specific barriers prior to the Roundtable but IFAA will arrange interviews so this information can be added to this ongoing data collection effort.

The most common patient reported barriers to engagement, *based on patients who are not typically “at the table”*, included:

- Uncertain how to get involved and to what level could they be involved without education
- Not interested in this level of engagement
- Concerned if they will look foolish
- Inability to travel or, even with ‘flexibility’ options, fear of committing when flares are unpredictable (*Tested in ACT Phase I*)

**Importance of Creating Subgroups of Patient Stakeholders.** As more and more patients are becoming educated in a variety of research protocols, so the roles between “patient”, “educated patient”, and “patient-researcher” is becoming blurred. Some Roundtable attendees suggested that as definitions of patient engagement expand, or new ones are defined, that the ‘roles’ of “patient” may also need to be established. This will be of significant importance as we consider value measurements, and begin to better understand how different levels of expertise can affect the outcomes. Furthermore, as stated by Ben Nowell, Global Healthy Living Foundation, “Understanding the benefits and contributions at these different levels of expertise could add benefit to patient engagement design long term.”

After reviewing barriers reported by all stakeholder groups, only one was mentioned by every stakeholder (other than Pharmaceutical Researchers, who currently have little if any direct contact with patients): *limitations due to disease activity*. *This was further explored in the following Discussion Question:*

**Discussion Question #3**

> When engaging patients in research, the one common barrier - regardless of stakeholder - involved working around flares/the unpredictability of disease. Thinking about those issues specifically, how do you/does your affiliation adjust for flexibility?

**Discussion Question #3 Conclusion:**

Almost all answers submitted mentioned *online/remote participation is ideal to navigate disease issues and promote flexibility*, particularly if a goal for future engagement is to include a larger sample. Also making certain to *ask the patients what they need* was equally as important.

**Additionally, those who have participated as Patient Advisors added:**
● If meetings are in person it’s important to offer rest periods, shifts, and even filling in for another patient if they need to take a break. - Niti Goel, GRAPPA

● It is important to consider not only the length of the day (for example 7:30 to 5pm is often too long for a patient, even with breaks), but also the time the meeting will start. Patients deal with severe stiffness that makes morning sometimes difficult. So an early start for most may require an additional hour earlier for the patient for warm up time, in addition to more time to get ready. - IFAA

● Patients have issues with energy so making sure there are plenty of snacks available (such as seeds, nuts, and other foods that provide natural energy) is important. - IFAA

● Some patients require a travel companion. If these patients are chosen for an in person meeting, it should be required to pay for both the patient and their travel assistant. – IFAA

In the ACT project specifically, patient engagement was predominantly done online, with careful consideration to flexibility (both in time commitment and to accommodate physical limitations), and was entirely designed by patients who had experience as PRPs or PCOR reviewers, and the Project Manager worked previously at a qualitative research center and is currently the sole patient reviewer at NIAMS/NIH.

Discussion Question #4

Do you have any other suggestions to help overcome barriers to patient engagement that have not been discussed here today?

Discussion Question #4 Conclusions:

● Publish a collective body of work that shows the value of PRP involvement, e.g., it has helped improve the research. Highlight the items that come to the forefront in research when patients are involved versus when they are not. - Niti Goel, GRAPPA

● Any type of patient-generated, patient-led research will help to advance research and funding, which we need because we are significantly underfunded in our diseases. This creates another box, another funding source. But defining it and then formalizing it is a necessary, important step. – Cheryl Koehn, PRP, President Arthritis Consumer Experts/JointHealth™

● Collect testimony from researcher peers – fellow researchers – who have engaged with patients. Example from an ACT LIVE! interview with Clayon Hamilton, Arthritis Research Canada (ARC): “I have had four patient partners now on my projects and it has improved the original idea to become much more advanced than we originally thought it would be.”
New Barriers Identified

**2016 2017**

ACT

New Barriers

- **Duplication of efforts = wasted research $$$**
- **“N of 1” results = lack of value measurements**
- **Lack of education about existing & newly defined terms & methods may disrupt value measurements**

100’s New Projects

New benefits & new barriers must be reported & distributed on a global level, so we can continue to develop stronger engagement efforts, measure value, & avoid a duplication of efforts.

**Duplication of Efforts**

As mentioned previously, as patient engagement efforts expand to new directions, ensuring those designing projects are aware of what has already been done is key to avoid a duplication of efforts, thus wasting time and research dollars. This was even demonstrated in the ACT project, as some of the original design included extensive interviews with various researchers to establish a list of the most pressing barriers to patient engagement; however, this was already done to a similar degree by the Clinical Trials Transformation Initiative in 2015. So instead of doing it again, we cited their research and built on their findings, which helped the ACT project develop more meaningful researcher interview questions during ACT Phase II (ACT LIVE!).

IFAA, while “at the table” as patients and actively participating in research projects, realized if we were close to duplicating efforts, others may be too. So we tested this theory during the Roundtable, where attendees were representative of those most involved in patient engagement work.

**Poll #1**

IFAA asked attendees if they were familiar with the CTTI project (*US based project, concluded 2015*). Of those who responded (n=18) just over 60% were familiar with the project, but the remaining were unfamiliar. Upon further review, of those unfamiliar, most were *not* US based.

**Poll #2**

This poll involved a project recently launched by Arthritis Research Canada called Assessing Meaningful Patient Engagement in Research Study/PEIRS (*Canadian based project, began late 2016*). Only 46% of those who responded (n=18) knew about this project. However, it is new so the findings have not been published; additionally, as seen in the prior poll, *those who did not reside in Canada were not as familiar with this project*.

**Polls #1 and #2 Conclusions**

We concluded that it is possible, even for those *most* engaged in this process, to duplicate efforts – particularly if important projects are unknown due to geographical barriers. Moving forward, particularly if we hope to build on past successes – and, in turn, build credibility and value for patient engagement methods – there is a need for global dissemination of results. Additionally:

- **There is a need for quantifiable results.** Most proof of benefit is done internally by comparing success to known measurement standards (example: “Clinical trials average 12-15 amendments; adding a patient group to the trial review, particularly by reviewing inclusion/exclusion criteria, reduced our trial amendments to 9, thus saving...”
time and money.") While this method benefits individual groups, these “N of 1” results fail to prove value for researchers and industry, who often require it to be demonstrated with quantifiable data. This will become even more important as we advance patient engagement efforts past typical non-pharmacologic methods (PRP) and into the patient-industry/drug development environment, which will require a large patient sample to quantify significant and robust data.

- **Failure to educate can be problematic for progress.** As stated earlier in reference to conferences, those new to patient engagement are not being properly educated regarding definitions of existing methods and how they are relevant (or not) to designing their projects. Additionally, as demonstrated in Polls #1 and #2, **even those most active in patient engagement in our own rheumatology community** may not be familiar with former or existing efforts. As patient engagement is still surging and branching off in new directions, it is our responsibility to ensure the concepts of involvement are understood and that pivotal efforts are tracked in a way so that duplication of efforts is avoided and so value can be recorded in a quantifiable manner.

- **New projects & innovative efforts.** Patient involvement in drug development has resulted in the need to find new ways for patients-pharmaceutical researchers-industry to collaborate differently than methods already established through prior methods of PRP and PCOR. With any new platform tested, we will discover new barriers and benefits that also must be recorded and sectioned to subgroups (pharmaceutical vs non-pharmaceutical researchers, for example) in order to continue forward with value measurements and so as engagement efforts continue to expand those new to the process have a foundation to build from and so efforts will not be duplicated.

**Next Steps**

There are many groups working to create guidance and standards of patient engagement in research, but none are specific to the rheumatology community. This matters because **our patient population has unique disease limitations, range of current expertise, and outcome preferences** that differ from other disease groups. **Identifying how patients have been - and are continuing to be - involved in rheumatology specific research as it continues to evolve can strengthen the development of new initiatives and increase the opportunity to measure their value within our community.**

- Create a history of patient engagement in rheumatology research that tracks concepts of involvement, guidance, and pivotal evolution in processes so those designing projects can build on successes, lessen the risk of duplicating efforts, and increase value measurements (by promoting longitudinal comparisons rather than N of 1 results). Doing this specifically as it relates to rheumatology projects can strengthen our impact and help stakeholders see the value associated with having our patients included in projects.

- Create a master log of known barriers and benefits to engagement, per stakeholder group and phase in the research continuum. Add new barriers and benefits as discovered.

- Expand “Patient” Stakeholder group to include subgroups, as now patients are involved from a novice level up to professional researcher status. Further determine how each level brings value.

We propose establishing a council of stakeholders who have been active in the rheumatology patient-included research - with patients who have been "at the table" at the lead - to begin working together to strengthen our community projects.
**Funding Statement**

The initial ACT project, which included this Roundtable event, was an award-winning project funded by Celgene. Special thanks to those who attended the Roundtable, and especially to those who contributed additionally to the commentary and post-session contribution to data. Together we can ensure the history of patient engagement is preserved and the future innovative ideas have a reference from which to build upon.

**Attendance (Live Meeting)**

*I=Industry, R=Researcher, NP Nonprofit, PRP=Patient Research Partner Working Group, P=Patient*

- Alison Hoens, ARC’s Arthritis Patient Advisory Board, BC Support Unit/SPOR (PRP, P)
- Annalisa Dialino-Felix, Medimmune (I)
- Ben Nowell, Global Healthy Living Foundation (NP, R)
- Cheryl Koehn, Arthritis Consumer Experts/Joint Health™ (NP, PRP, P)
- Clayon Hamilton, Arthritis Research Canada (R)
- Deeanna Quist, IFAA (NP, P)
- Ina Campbell, GRAPPA (PRP, P)
- Jennifer Horonjeff (R, PRP, P)
- Kaleb Michaud, National Data Bank for Rheumatic Diseases (NP, R, PRP, P)
- Kelly Conway, IFAA (NP, P)
- Kelly Franchetti, MAPI Group (CRO, P)
- Kirsten Lerstrøm, Lupus Europe (NP, P)
- Kirstin Bacani AstraZeneca (R, I)
- LaRita Jacobs, IFAA (NP, P)
- Maarten de Wit, OMERACT, PARE, EULAR (PRP, P)
- Michael Mallinson, Canadian Spondylitis Association (NP, P)
- Niti Goel, GRAPPA, Quintiles (R, PRP, CRO, P)
- Pamela Love, GRAPPA (PRP, R)
- Rebecca Schumacher, National Data Bank for Rheumatic Diseases (NP, R)
- Steven Brunette, Boehringer Ingelheim (I)
- Tami Brown, IFAA (NP, P)
- Valerie Powell, MAPI Group (CRO, R)
- Virginia Ladd, American Autoimmune Related Diseases Association/AARDA (NP, P)

**About IFAA**

IFAA is an international foundation that focuses only on the autoimmune and autoinflammatory diseases that include inflammatory arthritis as a major component in most patients. Led by persons living with these diseases, we use our experience as patients - in conjunction with professional backgrounds - to identify and solve issues that impact education/awareness, advocacy (public policy), and research.
individual differences in lifestyle, environment, and biology, researchers will uncover paths toward delivering precision medicine. [Link](https://www.nih.gov/research-training/allofus-research-program)


5. European Medicines Agency [Link](http://www.ema.europa.eu/ema/)

6. Innovative Medicines Initiative (IMI) [Link](http://www.imi.europa.eu/)

7. John Hopkins Center for Patient-Centered Outcomes Research in Rheumatology [Link](https://www.hopkinsrheumatology.org/specialty-clinics/cpcorr/)


10. OMERACT’s publication on Patients as Partners, 2016 [Link](http://www.omeract.org/pdf/OMERACT_patient_involvement.pdf)

11. Patient-Centered Drug Development in Oncology [Link](http://bmjopen.bmj.com/content/3/5/e002241)

12. PARE (EULAR’s Standing Committee for PRP) [Link](https://www.eular.org/myUploadData/files/PARE_Values_Principles_web.pdf)

13. PCORI’s Revised Definition of “Patient Centered Research Outcomes”, 2012 [Link](http://www.pcori.org/assets/PCOR-Revised-Definition-v2-042020121.pdf)

14. Strategy for Patient-Oriented Research (SPOR). Research that is done in partnership with patients, answers research questions that matter to patients, and aims to improve health care in Canada [Link](http://bcsupportunit.ca/); [Link](http://www.cihr-irsc.gc.ca/e/44000.html#a1.2)

15. US Food and Drug Administration [Link](https://www.fda.gov/)