Collaborating to support patient and public involvement in research

Monday 16th July 2018

ABPI Offices, London UK

Contents

Introduction

At the Patients First conference in March 2018, the AMRC and the ABPI both stated a commitment to working together to promote patient and public involvement (PPI) in medicines R&D. The conference was attended by some members of the Shared Learning Group on Involvement in Research (SLGIR) – a grouping of national charities with an interest in health and a commitment to actively involve patients in the research that they fund and support.

Group members met with ABPI early in 2018 and agreed to work together to plan a joint meeting.

Aims

The purpose of the meeting was to promote collaboration between charities and industry to support patient involvement. The aims of the meeting were:

- to share and appraise case studies of UK charities working with industry to support the involvement of patients in research
- to identify what needs to be in place to take this type of collaboration forward, what is already being developed and the priorities for action in the UK

Format and organisation

The meeting was organised and hosted by the ABPI and the Shared Learning Group on Involvement in Research (SLGIR). Representatives from the ABPI and SLGIR worked together to plan the meeting. The meeting took the form of a workshop, with presentations and group discussions.

Attendees

A list of attendees is included as Appendix One.

Agenda

12:30	Networking lunch
13:00	Welcome and introductions
13:15	What is already happening to support PPI in R&D?
	Group discussion
13:35	Exploring case studies: Looking at collaborations between pharmaceutical companies and charities
	Presentations followed by group discussion
14:30	Break
14:45	What do we need to do to improve collaborative working?
	Breakout group discussions

15:30	What would help move this agenda forward in the UK?
	Group discussion and prioritisation exercise
16.15	Close

What is already happening to support PPI in R&D?

The group discussed a number of programmes and initiatives that are already in place to support patient involvement in medicines R&D (referred to as 'patient engagement' in Europe and the US):

Patient Focused Medicines Development (PMFD)

PFMD is an international consortium of pharmaceutical companies, regulators, patient organisations and patients coming together to drive patient and public involvement in medicines R&D. PFMD was established in October 2015 and currently has 26 members. PFMD have developed a number of tools for stakeholders:

- Synapse – a searchable online tool to identify patient engagement initiatives internationally. Includes case studies and examples of programmes supporting patient involvement
- Patient Engagement Quality Guidance (PEQG) – a set of seven quality criteria that can be used to plan or assess patient involvement activities
- Book of Good Practices – a selection of examples of patient involvement that can be considered good practice according to the PEQG

PFMD are working with MPE and WECAN to develop guidance on legal agreements between pharmaceutical companies and patient organisations, which will include template contracts and a toolbox.

European Patients' Academy on Therapeutic Intervention (EUPATI)

EUPATI is a pan-European consortium focused on educating and training people affected by health conditions to ensure they can meaningfully contribute to medicines development. EUPATI currently offer two levels of training for patient advocates:

- EUPATI Patient Expert Training Course – a comprehensive 14-month programme offering expert-level training in medicines R&D
- EUPATI Toolbox – an online resource of information about medicines R&D

A one-day 'introduction to medicines R&D' training course is also currently in development, due to be piloted in Autumn 2018.

EUPATI also have guidance documents on patient involvement in pharmaceutical industry-led medicines R&D, ethics committees, regulatory processes and health technology assessments (HTA).

Patients Active in Research and Dialogues for an Improved Generation of Medicines (PARADIGM)

PARADIGM is a 30-month IMI project which aims to advance meaningful patient involvement in the lifecycle of medicines development by developing processes and tools to support patient involvement in three key points in the process: research priority setting, design of clinical trials and early dialogue. PARADIGM has 34 consortium members.

The first step of the project is a comprehensive survey to a wide range of stakeholders to understand the current landscape and identify gaps. The project will develop a set of tools and practices that support mainstream patient involvement, along with validated metrics which can be used to evidence the impact of involvement.

Other initiatives/projects:

- **PREFER** is a 5-year IMI project which aims to develop recommendations for industry, Regulatory Authorities and HTA bodies on how to use patient-preference studies to support decision making. The project is entering year 3 and is currently developing case studies of best practice.
- NIHR are carrying out a one-year project to define their offer for enabling patient involvement within pharmaceutical R&D. A soft launch will take place in October.
- **eyeforpharma** provides a platform for pharmaceutical representatives, patient advocacy groups and other key stakeholders to exchange ideas and stay up-to-date with current trends and practices. They have produced some materials around meaningful patient involvement.
- Regulatory bodies – both the EMA and FDA support active patient involvement/engagement in their activities.

Exploring case studies: Looking at collaborations between pharmaceutical companies and charities

Two case studies were presented as examples of what can be achieved when pharmaceutical companies collaborate with charities to carry out patient involvement. Presenters shared the processes involved, enablers and challenges of each collaboration.

- Arthritis UK and Pfizer (Slides are attached as Appendix Two)
- Parkinson's UK and UCB (Slides are attached as Appendix Three)

The case studies helped to highlight key areas where further support and guidance is needed to support this type of collaborative working. The presentations stimulated a number of questions and interesting discussions. Key points included:

- The level of experience of those involved – some people involved are patient experts, others are not. In the case studies presented, those who attended self-selected to be involved – we need to be aware of potential bias.

- It's important to ensure those involved are representative of the broader group of potential patients (although this is also an issue for other areas of the research process, e.g., participation). There is recognition that there needs to be more diversity in PPI. However, we need to start somewhere. Openness and transparency about who is involved is important.
- Ultimately people are there to give their insight as someone affected by the health condition, and this is very valuable.
- It is important to also involve people who aren't patients themselves, including carers and family members
- It would be useful to have Key Opinion Leaders (KOLs) involved in meetings with patients too so they can hear views of patients first hand
- It's important to bear in mind dynamics when bringing different groups of people together – be mindful that the presence of some groups could influence what people feel like they can say
- We need to be clear about the purpose of this work – is it to improve how patient organisations and industry work together to support patient involvement in R&D (with patient organisation acting as the broker), or is the purpose to improve patient involvement in R&D?
- The ABPI Code is often seen as a barrier to patient involvement and patient organisation-industry collaboration

What do we need to do to improve collaborative working?

Charities and industry split into two groups to discuss what each sector needs to improve collaborative working. Both groups discussed both their own needs and the needs of the other sector. The group then reconvened to share ideas and undertook a prioritisation exercise by voting on which topics they thought were most important in moving the agenda forward in the UK. The number of votes is indicated in brackets, and the top two priorities from each sector are highlighted in bold.

What does industry need in order to effectively work with charities?

- **Case studies of where patient involvement has made a difference to commercial research that companies can use internally – evidence base (13)**
- **Support to understand the code of practice in relation to patient involvement – blueprint of what can be done (10)**
- Clear metrics – how involvement has added value (5)
- Clarity of the benefits of working with a research charity and role of patient organisation in facilitating involvement (4)
- Short, quick process from first engagement to end results (3)
- Training for legal and compliance teams on the code and how to work with patient organisations (1)
- Knowing who to contact (patients, patient organisations) – single entry point
- To be more focused on what we are asking
- To understand what global medicine needs

- Charities to state whether they will or will not work with pharma on PPI – check list of requirements/purpose
- To understand that patients want to be involved
- To help think about whether the charity should be paid and why
- To be clear about what information can/can't be shared, including feedback
- Central place to share examples of involvement and impact, including what industry are getting out of it and why they are doing it
- An understanding of charities' processes

What do charities need in order to work effectively with industry?

- **Guidance on policy positions in order to support transparency about how charities are working with industry – including payments, e.g. should charities take money? If so, why? What for? (11)**
- **A toolkit for working with industry, including an overview of research and development processes, template contracts, guidance on the code of practice and guidance on compliance and legal (9)**
- Support to be able to gather a diverse selection of patients and represent a spread of patient types with lived experience – diversification (3)
- Single point of contact in pharma (1)
- To be able to talk to other charities and learn from their experiences of working with industry – how they did it, lessons learned, what advice they would give etc. Not necessarily case studies, but actual conversations
- Clear objectives from industry about why they are involving patients – understanding of motives
- Shared clarity of purpose about why industry are involving patients – overall and for each project
- Risk management strategy
- Understanding of the offer from pharma – including benefits for patients
- To be able to maintain their independence
- Position on working with other charities

Addressing priorities: what would help move this agenda forward in the UK?

1 Develop a toolkit for working with industry

The toolkit will provide information and guidance for patient organisations on key aspects of working with industry. Information needs to be available in one single place for people to access. The toolkit can be a work in progress – there are some aspects that require more work before they can be included in the toolkit, but we can make a start now.

Next steps: SLGIR to capture learning from existing collaborations. This will form the basis of the toolkit. **By 30/9/18**

2 Develop policy guidance for patient organisations

Charities need guidance on key policy elements, including accepting payments. It was felt that this needs to be led by the AMRC so it will reach as many people as possible, with the support of ABPI and in partnership with charities and the SLGIR. Even with involvement of AMRC there is a danger of missing some groups e.g., patient advocacy groups. The group suggested that any guidance developed be shared with other organisations (e.g. National Voices).

Next steps: ABPI to discuss with AMRC. **By 30/9/18**

3 Clarification of code of practice in relation to patient involvement

There needs to be clarification of what is allowed under the code of practice and examples of what you can do. We need to join up conversations with ABPI Code Refresh.

Next steps: ABPI to involve SLGIR members in the review of the Code. **By 30/9/18**

4 Develop case studies for use by industry which demonstrate impact of PPI

These case studies need to be detailed and clearly demonstrate the value and impact of patient involvement for industry, and the benefits of working with patient organisations. They should include 'light bulb moments' – statements of the obvious/common sense are not sufficient. It was felt that case studies currently available lack the detail that companies need to demonstrate value.

Next steps: Parkinson's UK and Arthritis Research UK to work with Pfizer and UCB to write up case studies. **By 30/9/18**
ABPI to scope what information will be available from PARADIGM. **By 30/9/18**

Summary

The day ended with a discussion and some general comments:

- It is important to bear in mind that companies work globally
- Embedding patient involvement requires a cultural shift in the way industry work
- It can be difficult within a company to get people to understand the added value
- At present, patient involvement for many companies is a case of trial and error – seeing what works and what doesn't. We are all still learning.
- From the charity perspective, there is a need to differentiate between case studies (in-depth descriptions of experience) and impact reports (shorter, punchier)
- The goals for patient involvement need to be clear – there is still uncertainty within industry about why involvement is important. Goals should drive the evidence base, rather than the other way around.

- Shared purpose which is transparent to all involved is key.

Appendices

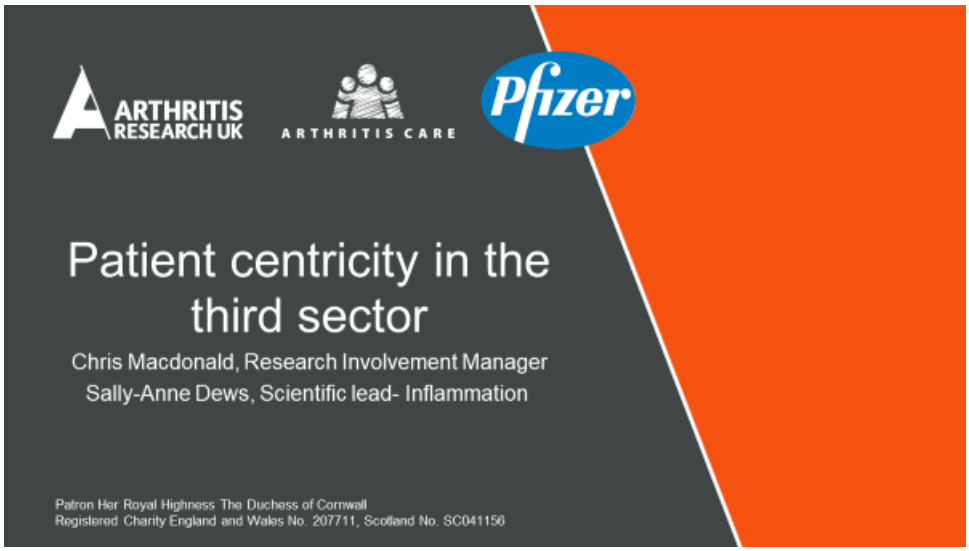
Appendix One – Attendees

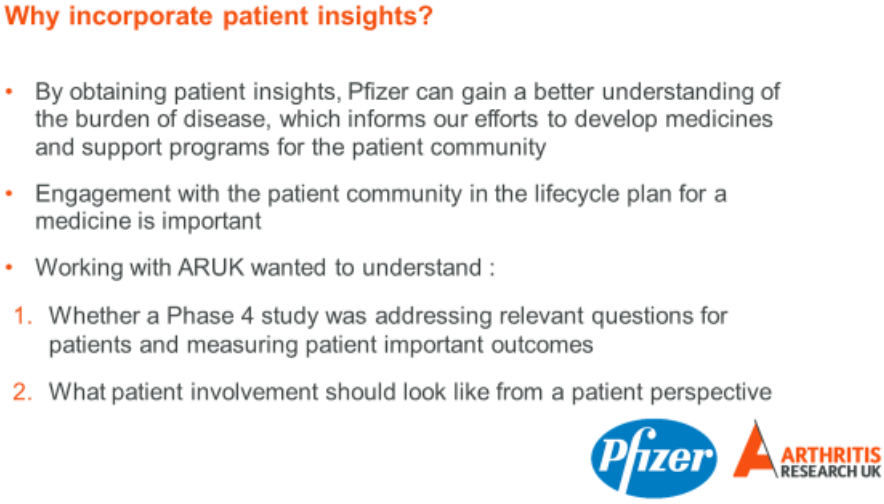
Name	Organisation
Harriet Adams	ABPI
Lorna Allen	Cystic Fibrosis Trust
Sally-Anne Dews	Pfizer
Aurelie Duces	Bristol-Myers Squibb
Jackie Dudley	Covance
Andrew Foxley	AstraZeneca
Andrew Garvey	GlaxoSmithKline
Sophie Gillmore	AstraZeneca
Katherine Guerin	Health Research Authority
Bec Hanley	Shared Learning Group on Involvement
Ali Hansford	ABPI
Teresa Klinowska	AstraZeneca
Nicole Lyons	Breast Cancer Now
Chris Macdonald	Arthritis Research UK
Claire Nolan	Parkinson's UK
Priya Patel	ABPI
Berkeley Phillips	Pfizer
Sheuli Porkess	ABPI
Natasha Ratcliffe	Parkinson's UK
Clare Spooner	Servier
Richard Stephens	National Cancer Research Institute
Kate Trenham	UCB
Willian van t'Hoff	NIHR
Caroline Wijnbladh	Asthma UK

Apologies:

AMRC
MSD

Appendix Two: Arthritis UK and Pfizer





Patient Insight Partners

People with arthritis will be involved in every stage research decision

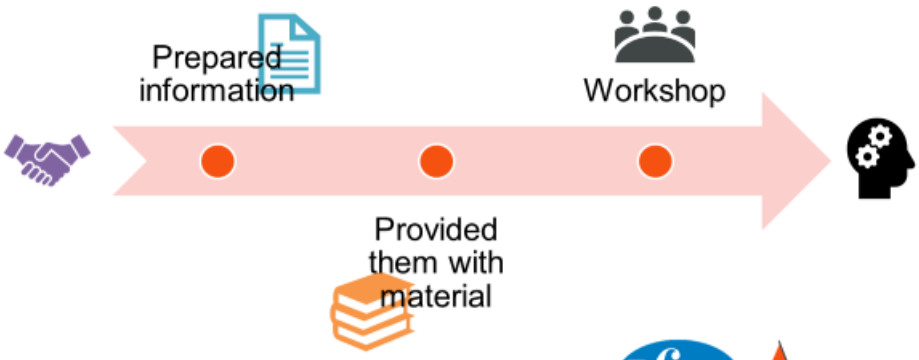
Pfizer ARTHRITIS RESEARCH UK

Main objectives

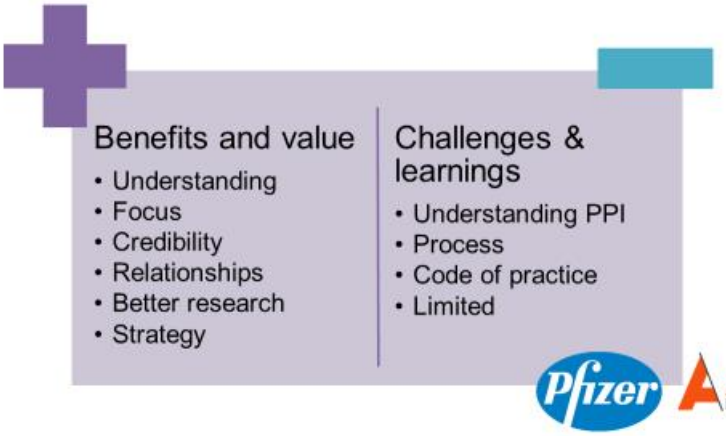
1. Understand the burden of rheumatoid arthritis and the needs of patients
2. To gather insights around the 'patient journey' required in a clinical study.
3. To gather feedback on:
   - Proposed study design
   - Study primary and secondary objectives
   - Patient reported outcomes measures (PROMs)
   - Study assessments
4. To understand the future role of patient involvement

Pfizer ARTHRITIS RESEARCH UK

How we involved people with arthritis



Collaboration in patient centricity



Key learnings in collaborative

Be legitimate and manage expectations

Be clear with objectives and focus discussion

Start early in trial development and continue throughout

Wider implications on research strategy

Patient insight partners offer very valuable insights and can contribute significant learnings:

- Relevance of outcome measures
- Feasibility of study assesments
- What effects willingness to participate/stay in trial

Feedback is important

Appendix Three: Parkinson's UK and UCB

Clinical Trial Development Workshop
Parkinson's UK and UCB Pharma
Collaboration Feedback

Inspired by patients. Driven by science.

PARKINSON'S UK
CHANGE ATTITUDES.
FIND A CURE.
JOIN US.

Funding for this collaboration was split accordingly: UCB: Venue, catering, reimbursement for volunteers time & medical writer. Parkinson's UK: Volunteers travel and expenses and external facilitator

Date of Preparation July 2018 UK/18NU0026

The purpose

- Help us to bring us better treatments and a cure, faster
- Expanding our support into the development of new medicines for Parkinson's

PARKINSON'S UK
CHANGE ATTITUDES.
FIND A CURE.
JOIN US.

The purpose

- To get input from people with lived experience of Parkinson's about their thoughts and/or experiences of participating in clinical trials
- To improve the design of the trial to maximise appropriateness, acceptability and success

Inspired by patients. Driven by science.

Clinical Trial Workshop - What we did

Joint working with external partners to plan, organise, deliver and evaluate a 1-day workshop with people with Parkinson's which entailed:

- **Agreeing objectives, draft agenda, numbers, criteria for participation**
- **Creating advert, pre-read materials (to include info on clinical trials, the importance of collaboration and an introduction to this research)**
- **Recruitment of 15 people affected by early stage Parkinson's**
- **Preparing the participants**
- **Deliver and evaluate workshop with UCB**

Workshop Discussions

- **Q&A with participant about clinical trial experiences**
- **Overview of UCB's new clinical trial**
- **What motivates people to take part in clinical trials?**
- **What would people like to know about a study?**
- **How do people feel about using technology as part of clinical trials?**
- **Thoughts on specific study tests, and intake and packaging of study medication**

Next steps

- **Feedback to participants/Internally**
- **Developing trial taking into account insights gleaned**
- **Continued dialogue via a Patient panel?**

The meeting was very well organised and the tone of the 'meeting' was rather like a Parkinson's family get-together. I felt at home and was really eager to meet and talk with everyone.
First class meeting, well moderated, kept on track and relevant to the subjects to be discussed. Lovely mix of PWP, researchers and medics all of whom were seriously interested in our discussions. A really uplifting experience.
Really enjoyable . Professional . Well put together
It was very thought out and Bec was doing a great job with the time keeping of the meeting.
Extremely well organised and Bec did a great job of moving the meeting forward on time.
OI's Great to have a voice
Very welcoming. I felt listened to and that my contributions were valued by all involved.
Informative and very participatory.

1. How would you rate the meeting overall? (1 = poor, 10 = excellent)

	1	2	3	4	5	6	7	8	9	10	Response Total
	0.0% (0)	0.0% (0)	0.0% (0)	0.0% (0)	0.0% (0)	0.0% (0)	9.1% (1)	9.1% (1)	0.0% (0)	81.8% (9)	11
										answered	11
										skipped	0

Participant feedback

8. Did you feel that your comments, experiences and suggestions were listened to and valued?

		Response Percent	Response Total
1	Yes	100.00%	11
2	No	0.00%	0
3	I don't know	0.00%	0
Analysis	Mean: 1 Std. Deviation: 0 Satisfaction Rate: 0 Variance: 0 Std. Error: 0	answered	11
		skipped	0

5.1. Your knowledge of clinical trials

		Response Percent	Response Total
1	1	0.0%	0
2	2	0.0%	0
3	3	18.2%	2
4	4	36.4%	4
5	5	45.5%	5
Analysis	Mean: 4.27 Std. Deviation: 0.75 Satisfaction Rate: 81.82 Variance: 0.56 Std. Error: 0.23	answered	11

3. Did you feel adequately prepared to contribute to the workshop?

		Response Percent	Response Total
1	Yes	100.00%	11
2	No	0.00%	0
3	I don't know	0.00%	0
Analysis	Mean: 1 Std. Deviation: 0 Satisfaction Rate: 0 Variance: 0 Std. Error: 0	answered	11
		skipped	0

5.2. Your confidence in working with researchers and offering your perspective to shape Parkinson's research and clinical trials

		Response Percent	Response Total
1	1	0.0%	0
2	2	0.0%	0
3	3	0.0%	0
4	4	36.4%	4
5	5	63.6%	7
Analysis	Mean: 4.64 Std. Deviation: 0.48 Satisfaction Rate: 90.91 Variance: 0.23 Std. Error: 0.15	answered	11

Impact

Parkinson's UK

- Helped us learn more about how pharma works and how we can support pharma and patients to work together
- Allowed us to adapt and develop our programme to more easily enable us to work with pharma
- Helped us to pilot how this kind of partnership can work
- Enhanced the reputation and credibility of our programme

For the volunteers

- Learned more about clinical trials
- Understood how they could contribute towards the development of new treatments
- Brought a sense of community and shared purpose – not them and us
- Gave them hope

UCB

- Helped us understand more fully how patient groups work to facilitate smoother engagement in the future
- Initiated discussions for internal restructure to support these collaborations
- Helped us demonstrate internally the value of gaining the patient perspective at the earliest stage possible in the medicine development lifecycle
- Feedback has already been incorporated in the clinical team's work in the study design

How UCB is using the information from the collaborative workshop?

1. Inform the study design and conduct of the upcoming Ph2 study:

- How to make study visits more patient-friendly
- How to adjust study visit schedules to patients' lives
- How to take into account patients' concerns around investigations such as lumbar punctures, brain imaging, blood sampling

2. Ensuring access to the study drug to everyone once the study has finished

3. Drug formulation and the Smart packaging have been reviewed

4. Study will be publicised on the right websites once it is open for recruitment (Parkinson's UK website, MJFF…)

5. Improve the quality of information materials;

- Design of information leaflets
- Short video clips
- Clear explanations of the study drug
- Study procedures risks
- Communication in case of unexpected events e.g. such as early termination of the study

Challenges

- Maintaining independence
- Paying patients
- Contracts
  - Language
  - IP
  - Communication
  - Publication
  - Delays
- Compliance process
  - Setting up contracts with patients – administrative burden and language/additions
  - All external documentation to be approved by compliance
- Time consuming – planning meetings, recruitment, preparing patients/carers
- Many logistical & organisational challenges,
  - On the surface, it seems simple, but is far more complex
  - There is no project manager in place to deal with these workshops.

Lessons learned

- It's hard but it is worth it!
- Approaching contracts earlier and getting Legal teams together
- Turn around times from Pharma can be tight and lead to stress
- Sufficient resource and buy in is needed from all sides to support this work
- Working with external partners to facilitate and capture the discussions is essential for good quality, meaningful and impactful discussions
- Streamlining communication process
- Work with people affected by Parkinson's to plan PPI activities
- Be clearer that involvement doesn't mean access to trial
- A patient panel would be beneficial to avoid separate contracts and aid continuous, quicker and smoother communications
- Introductory calls helped the volunteers feel like they were coming into a more personal experience
- Patient groups skilled in translating Pharma materials in language that resonates better with volunteers
- Pre reads help streamline the process