



Incorporating Patient Perspectives in Clinical Trial Design and Research

A White Paper by the Science of Patient Input (SPI) Program
of the Medical Device Innovation Consortium (MDIC)

February 25, 2020
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PREFACE

Parkinson's patients Margaret Sheehan and Anne Cohn Donnelly, who participated as Patient Scientists on the MDIC Patient Centered Outcomes Research project team along with representatives from the Michael J. Fox Foundation for Parkinson's Research (MJFF), the U.S. Food and Drug Administration (FDA), and MIT,¹ provide their perspectives on how clinical researchers can best engage with patients. Their thoughtful advice provides guideposts for how clinical trial sponsors can effectively engage patients as partners throughout the clinical trial process. We commend the authors for providing the clinical research and medical product community with their insightful and impactful recommendations. Greater engagement of patients in the design and conduct of clinical research is of urgent necessity. Donnelly and Sheehan remind us that clinical researchers, physicians, industry sponsors, and – most importantly – patients will only benefit from including the patient voice early and often throughout the therapeutic development process.

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¹ MDIC Patient Centered Outcomes Research Project page. <https://mdic.org/project/patient-centered-outcomes-research/>. (2017).



INTRODUCTION

Patients have been, and always will be, central to the conduct of clinical trials and related medical research as the subjects of that work. Until recently though, researchers have not routinely included patients in the design of clinical trials, whether it be prioritizing the research objectives and outcomes based on importance to patients, the design of the research to be patient-focused, interpretation of findings and access to patients' own data, or in determining whether a clinical trial was a success or a failure. Even as patients have been increasingly engaged in their own care, patient involvement in all aspects of clinical trial research has evolved more slowly.² In short, clinical research has been mainly done *to* and *for* patients but not necessarily *with* them.

In the now rapidly evolving field of patient engagement in clinical research, the role of the patient is changing "from passenger to co-pilot."³ Many unanswered questions remain, however, about patient involvement in clinical research. Should patients have a voice in the design and interpretation of clinical trials? If so, how big a voice? In what ways should researchers include that voice? Where can patients be helpful? Where are patients essential? Should patients be treated as equal partners in research? What would constitute best practices for involving patients? Why should companies and other funders of clinical research invest the time and money in partnering with patients?

Over the past several years, we the authors – both Parkinson's patients – had the opportunity to serve as Patient Scientists on a study conducted by the United States Food and Drug Administration (FDA), the Medical Device Innovation Consortium (MDIC), The Michael J. Fox Foundation for Parkinson's Research (MJFF), and the Massachusetts Institute of Technology (MIT) focused on incorporating patient preferences in clinical trials.⁴⁻⁷ We served as full partners in the research, experiencing firsthand all aspects of the research process. This paper draws on that experience, as well as the extant literature, to address these questions of patient engagement in clinical research, and to offer directly to clinical researchers some patient perspectives on how to do it well.⁸

² Laurance, J., S. Henderson et al., "Patient Engagement: Four Case Studies That Highlight the Potential For Improved Health Outcomes And Reduced Costs," *Health Affairs*, (2014) VOL. 33 NO. 9

³ Biotechnology Innovation Organization, "Key Considerations in Integrating Patient Preferences in Drug Development." BIO (2016).

⁴ Ho, M., Saha, A., et al., "A Framework for Incorporating Patient Preferences Regarding Benefits and Risks into Regulatory Assessment of Medical Technologies." *Value in Health* 19 (2016). 746-750.

⁵ Irony, T., Ho, M., et al., "Incorporating Patient Preferences Into Medical Device Benefit-Risk Assessments." *Statistics in Biopharmaceutical Research*. (2016)

⁶ Chaudhuri, S. E., Ho, M.P. et al., "Patient Centered Clinical Trials." *Drug Discovery Today* (2017).

⁷ Isakov, L., Lo, A., et al., "Is the FDA Too Conservative or Too Aggressive: A Bayesian Decision Analysis of Clinical Trial Design." *Social Science Research Network* (2017).

⁸ Levitan, B., K. Getz, et al., "Assessing the Financial Value of Patient Engagement." *Therapeutic Innovation & Regulatory Science*, Sage Journal (2017)



WHY SHOULD PATIENTS BE INVOLVED IN YOUR CLINICAL TRIAL DESIGN AND RESEARCH?

Clinical researchers undoubtedly have noticed over the past few decades that they have been encouraged (if not required) through policy, regulation, funding panels, and purveyors of best practices to engage patients in their research.⁹⁻¹² Some say there has been a patient revolution over the last several decades.¹³ Whether a revolution or not, clinical researchers who have actively engaged patients have reported that their work is of higher quality, as well as more rewarding, enjoyable, and meaningful.¹⁴⁻²¹ Many groups have reported that engaging patients has resulted in cost savings as a consequence of faster completion of the clinical trials.²²⁻²⁴ Rather than being viewed as a burden, engagement should be viewed as an approach that can result in tangible financial value.

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- ⁹ Patient-Centered Outcomes Research Initiative (PCORI), "Better Research through Engagement." <https://www.pcori.org/sites/default/files/PCORI-Better-Research-Through-Engagement.pdf>. (2019)
 - ¹⁰ Patient-Centered Outcomes Research Initiative (PCORI), "Engagement Strategies, Challenges and Resources." <https://www.pcori.org/sites/default/files/PCORI-Patient-Stakeholder-Engagement-Challenges-Strategies-Resources-Presentation-Slides-120517.pdf>. (2017)
 - ¹¹ Clinical Trials Transformation Initiative (CTTI). CTTI Recommendations on Effective Engagement with Patient Groups Around Clinical Trials. <https://www.ctti-clinicaltrials.org/briefing-room/recommendations/ctti-recommendations-effective-engagement-patient-groups-around>. (2015).
 - ¹² Patient-Centered Outcomes Research Institute (PCORI), "Highlights of PCORI-Funded Research Results." <https://www.pcori.org/research-results-home>. (2019)
 - ¹³ Duffett, L., "Patient engagement: What partnering with patient in research is all about," Elsevier Press, (2013) 150: pp 113-120
 - ¹⁴ Kuehn C., Selig W., "Literature Review: Patient Engagement Clinical Trials," Medical Device Innovation Consortium (MDIC), (2019)
 - ¹⁵ Patient-Centered Outcomes Research Initiative (PCORI), "Better Research through Engagement." <https://www.pcori.org/sites/default/files/PCORI-Better-Research-Through-Engagement.pdf>. (2019)
 - ¹⁶ Patient-Centered Outcomes Research Initiative (PCORI), "Engagement Strategies, Challenges and Resources." <https://www.pcori.org/sites/default/files/PCORI-Patient-Stakeholder-Engagement-Challenges-Strategies-Resources-Presentation-Slides-120517.pdf>. (2017)
 - ¹⁷ Clinical Trials Transformation Initiative (CTTI). CTTI Recommendations on Effective Engagement with Patient Groups Around Clinical Trials. <https://www.ctti-clinicaltrials.org/briefing-room/recommendations/ctti-recommendations-effective-engagement-patient-groups-around>. (2015).
 - ¹⁸ Patient-Centered Outcomes Research Institute (PCORI), "Highlights of PCORI-Funded Research Results." <https://www.pcori.org/research-results-home>. (2019)
 - ¹⁹ Tenaerts, P., L. Madre, M. Landray, "A decade of the Clinical Trials Transformation Initiative: What have we accomplished? What have we learned?" Clinical Trials Transformation Initiative, (2018)
 - ²⁰ O'Daly, S., S. Dowd, et al., "Incorporating Patient Input into Clinical Trials." *Sleep Medicine* (2017) 40(Supplement 1): e243-e244.
 - ²¹ Cheung, P. P., M. De Wit, et al., "Recommendations for the Involvement of Patient Research Partners (PRP) in OMERACT Working Groups. A Report from the OMERACT 2014 Working Group on PRP." *Journal of Rheumatology* (2016) 43(1): 187-193.
 - ²² Levitan, B., K. Getz, et al., "Assessing the Financial Value of Patient Engagement." *Therapeutic Innovation & Regulatory Science*, Sage Journal (2017)
 - ²³ Laurance, J., S. Henderson et al., "Patient Engagement: Four Case Studies That Highlight the Potential For Improved Health Outcomes And Reduced Costs," *Health Affairs*, (2014) VOL. 33 NO. 9
 - ²⁴ Sacristan J. A., A. Aquaron et al., "Patient involvement in clinical research: why, when, and how," Dove Press, (2016)

Patients offer a perspective not replicable in a research environment – living with the medical condition being studied. Patients can help bring the clinical definition of a disease to life, humanizing the condition, the research focus, and the impact of any conclusions. Patients provide invaluable insight into what is most important to those the research is ultimately designed to help and how to most effectively conduct that research.

There are a multitude of practical reasons to include patients in all stages of your research, including:²⁵⁻³²

- Patients are a great resource for generating new research ideas.
- Patients offer insight about research priorities, reducing inefficiencies in the research and helping to inform investment decisions.
- Patients can help increase the relevance of a clinical study by commenting on the importance of the study's focus to those affected by the disease.
- Patients can help improve the quality of clinical research by suggesting study designs that are informed by patient experiences and sensitive to patients' living situations, making it more likely patients will participate in and complete studies.
- Patients can offer insights that improve the outcome measures by suggesting measures that are reliable and important to patients.
- Patients can assist with creating lay-friendly clinical study materials that are patient-accessible.
- Patients can provide input on risks they would be willing to take to achieve a benefit.
- Patients can help enrich the ways clinical trial data is analyzed and interpreted.
- Patients can help determine which findings are most important to patients, help disseminate the research findings, and advocate for their use to relevant stakeholders.
- Finally, patients can provide access to communities of fellow patients to participate in clinical trials to help with recruitment and retention (perhaps the biggest challenge in the successful implementation and completion of clinical trials).^{33 34}

There are ethical considerations as well. People whose lives will be most affected should have a voice in the research, from prioritizing design considerations to interpreting results.

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- ²⁵ Patient-Centered Outcomes Research Initiative (PCORI), "Better Research through Engagement." <https://www.pcori.org/sites/default/files/PCORI-Better-Research-Through-Engagement.pdf>. (2019)
- ²⁶ Patient-Centered Outcomes Research Initiative (PCORI), "Engagement Strategies, Challenges and Resources." <https://www.pcori.org/sites/default/files/PCORI-Patient-Stakeholder-Engagement-Challenges-Strategies-Resources-Presentation-Slides-120517.pdf>. (2017)
- ²⁷ Clinical Trials Transformation Initiative (CTTI). CTTI Recommendations on Effective Engagement with Patient Groups Around Clinical Trials. <https://www.ctti-clinicaltrials.org/briefing-room/recommendations/ctti-recommendations-effective-engagement-patient-groups-around>. (2015).
- ²⁸ Patient-Centered Outcomes Research Institute (PCORI), "Highlights of PCORI-Funded Research Results." <https://www.pcori.org/research-results-home>. (2019)
- ²⁹ Concannon, T. W., P. Meissner, et al., "A New Taxonomy for Stakeholder Engagement in Patient-centered Outcomes Research." *Journal of General Internal Medicine* (2012) 27(8): 985-991.
- ³⁰ Anderson, M. "On the Path to a Science of Patient Input." *Science Translational Medicine* (2016) 8(336): 336ps311.
- ³¹ Cheung, P. P., M. De Wit, et al., "Recommendations for the Involvement of Patient Research Partners (PRP) in OMERACT Working Groups. A Report from the OMERACT 2014 Working Group on PRP." *Journal of Rheumatology* (2016) 43(1): 187-193.
- ³² National Health Council, "Dialogue Advancing Meaningful Patient Engagement in Research Development, and Review of Drugs." National Health Council & Genetic Alliance. (2015)
- ³³ Kadan, R.A., Borde, S. U., et al. "Challenges in Recruitment and Retention of Clinical Trial Subjects." (2016) *Perspectives in Clinical Research* 7(3): 137 – 143.
- ³⁴ Varse, F., Janani L., et al. "Challenges in the design, conduct, analysis, and reporting in randomized clinical trial studies: A systematic review." (2019) *Med J Islam Repub Iran*. 33:37 doi: 10.34171/mjiri.33.37.

PATIENTS AS PARTNERS

How will patients be involved in the clinical research process?

Figuring out how to involve patients in the clinical research process (e.g., what role or roles will they play) is a critical first step to engaging with patients and will largely determine the number of patients you will need to have as partners on the clinical research team.³⁵⁻³⁸ It may work best to engage only one patient team throughout the project or engage several patient teams (which may or may not have overlapping members) for various roles. If the clinical research is complex or will involve commitments in excess of a year, then the research team should consider relying on multiple teams of Patient Partners with different levels of involvement and commitment of effort and time.^{39, 40}

The options include:

- **Engage a small number of patients to be involved in all aspects of the clinical research and for the entire length of the project.** We refer to this small group of highly engaged patients as Patient Scientists. In addition to providing information and guidance, these patients can be spokespeople, advocates, and liaisons for the clinical study and research team and may be considered full team members. It may be helpful to the integration process if the Patient Scientists already know each other before becoming involved with the clinical study.
- **Rely on a core patient group or committee to consult with on a regular basis regarding fundamental issues such as patient preferences, priorities, and tolerance for risk.** This patient group need not participate in each major step of the project, but should receive regular reports, and should be consulted with respect to decisions that have implications for patients. This patient group should be diverse enough (e.g., age, lifestyle, progress of the disease) to speak for the broader patient community.
- **Involve individual patients for specific steps within the clinical research study, such as background data and beta testing.** These patients may only be involved in a single task and/or independently of each other, such as giving phone interviews, completing questionnaires, or participating in focus groups.
- **Engage a large number of patients to provide data for any purpose for which significant patient participation is critical to validate results and conclusions.** Survey respondents are an example of this sort of group – commitment per patient is minimal, but the volume of responses creates value in the form of breadth and variety of responses.

Clinical researchers should determine which approach is most appropriate before starting the search for patients.

³⁵ Clinical Trials Transformation Initiative (CTTI), "Recommendations: Effective Engagement with Patient Groups Around Clinical Trials." Clinical Trials Transformation Initiative (2015)

³⁶ Huang, G., Bull, J. et al., "Clinical Trials Recruitment Planning: A proposed Framework." Contemporary Clinical Trials (March 2018): 74-79.

³⁷ National Health Council, "Integrating the Patient into the Drug Development Process: Developing FDA Guidance." National Health Council & Genetic Alliance. (2016)

³⁸ National Health Council, "Patient Centered Value Model Rubric," National Health Council, (2016).

³⁹ Hamilton, C. B., A.M. Hoens, et al., "An Empirically Based conceptual framework for Fostering Meaningful Patient Engagement in Research," Health Expectations (2018) 21 (1): 396-406

⁴⁰ Haywood, K., A. Lyddiatt, et al., "Establishing the values for patient engagement (PE) in health-related quality of Life Research: An International, Multiple-Stakeholder Perspective." Quality of Life Research (2017) 26(6): 1393-1404



Where can you find patients to participate with you?

Clinicians have historically served as proxies for their patients. However, consulting only with doctors about what's important to patients is no longer good enough. Even the most knowledgeable clinicians cannot speak for their patients most effectively. They don't know the daily experiences of their patients who are living with a disease, disorder, or medical condition. Additionally, in this age of connectivity, sharing, and communication, obstacles to reaching out to patients directly have been removed.

The best places to find patients will depend on the specific needs of the project. Patient advisory committees of foundations, research institutions, and advocacy groups are a good source of informed, committed patients for Patient Scientists or core patient groups. For focus groups, try tapping into support groups (including online support groups), research institutions, clinicians' offices, and classes or programs designed for people with the relevant condition. Conferences, online patient platforms, or web portals will provide a source of potential participants for large and diverse patient populations.

How can you successfully recruit patients to engage in the research process?⁴¹⁻⁴³

All Patients

Clinical researchers have an advantage when asking for help from patients because they share a common goal: they want to improve the lives of people living with the disease, disorder, or condition being studied. The more simply and compellingly researchers can make that point, the more positive the response will be.^{44, 45, 46, 47}

Tailor the pitch to the role for which patients are being recruited. In every case it will be useful to:

- Describe the problem and the goal of the project. Explain *what* the research will try to accomplish and *how* the researchers hope to accomplish it.
- Provide the broader context of which the clinical research is a part. Seeing the big picture will help patients understand why this particular research is important.
- Explain the impact the patients may and/or will have. Emphasize the importance of patients and the value of their participation.
- Be clear about the expectations of patient participants. Be specific in terms of time, travel, potential compensation, reading, reviewing, writing, and speaking obligations.
- Avoid acronyms and jargon, which create an insider versus outsider dynamic between the clinical researchers and patients.

⁴¹ Huang, G., Bull, J. et al., "Clinical Trials Recruitment Planning: A proposed Framework." *Contemporary Clinical Trials* (March 2018): 74-79.

⁴² Dewulf, L., "Patient Engagement by Pharma--Why and How? A framework for Compliant Patient Engagement." *Therapeutic Innovation & Regulatory Science*. (2015) Vol. 49(1) 9-16.

⁴³ Hamilton, C. B., A.M. Hoens, et al., "An Empirically Based conceptual framework for Fostering Meaningful Patient Engagement in Research," *Health Expectations* (2018) 21 (1): 396-40

⁴⁴ Patient-Centered Outcomes Research Initiative (PCORI), "Better Research through Engagement." <https://www.pcori.org/sites/default/files/PCORI-Better-Research-Through-Engagement.pdf>. (2019)

⁴⁵ Patient-Centered Outcomes Research Initiative (PCORI), "Engagement Strategies, Challenges and Resources." <https://www.pcori.org/sites/default/files/PCORI-Patient-Stakeholder-Engagement-Challenges-Strategies-Resources-Presentation-Slides-120517.pdf>. (2017)

⁴⁶ Clinical Trials Transformation Initiative (CTTI). CTTI Recommendations on Effective Engagement with Patient Groups Around Clinical Trials. <https://www.ctti-clinicaltrials.org/briefing-room/recommendations/ctti-recommendations-effective-engagement-patient-groups-around>. (2015).

⁴⁷ Patient-Centered Outcomes Research Institute (PCORI), "Highlights of PCORI-Funded Research Results." <https://www.pcori.org/research-results-home>. (2019)



Patients for the Clinical Research Team

A detailed recruiting pitch is most important for the patients who will become part of the clinical research team (the Patient Scientists).^{48, 49} These are the patients who will be the most deeply involved in the project, who may have the greatest impact on the project, and from whom you are asking the most. It is important to demonstrate an understanding of the importance of incorporating the patient perspective when making your pitch. Making this pitch in-person, if feasible, will reveal how much the clinical researchers are energized about and committed to the study. If an in-person meeting is not possible, consider a video meeting. Respect the prospective Patient Scientists' time, intelligence, and concerns. Give them the time and space to ask questions. Clinical researchers should explain why they are personally committed to including patient values and how they plan to accomplish this. Commit to asking for, listening to, and incorporating wherever possible the views and advice of patients at all stages as the clinical research moves forward.

Patients often feel frustrated by their condition and powerless to resolve it. Many would love the opportunity to *DO* something. Becoming a Patient Scientist offers patients a concrete way to have an impact. To most patients, letting them know what their involvement may mean will be music to their ears. Explain that their work will benefit the patient community and will be personally rewarding as well. Let them know that they will get satisfaction from being part of a team of experts working together to help others.

It is important to recognize that this type of work and setting (i.e., being part of a clinical research team) will likely be new to prospective Patient Scientists. They are being asked to work with PhDs, MDs, and highly trained people who they do not know and in an environment or place with which they may be uncomfortable at first. This is true for even very informed patients. To address any potential anxiety, provide some background about the way research projects are done, who the stakeholders are, and what obstacles the team may face. Humanize the experts with a quick summary of each team member's background and role. Make it clear that Patient Scientists will be full team members (if that is the case) and that their expertise in living with the condition is all they need to bring to the table. Reference the power of collaboration and the value of and need for different skills and perspectives on the project team.

Following these guidelines will encourage highly engaged patients to contribute extensively throughout the clinical trial process as full Patient Partners on your clinical research team.

⁴⁸ Shippee, N. D., J. P. Domecq Garces, et al., "Patient and Service User Engagement in Research: A Systematic Review and Synthesized Framework." *Health Expectations* (2015) 18(5): 1151-1166.

⁴⁹ Biotechnology Innovation Organization. "Key Considerations in Integrating Patient Preferences in Drug Development." BIO (2016).



When should patients be engaged in clinical research?^{50, 51}

Early...

Involving patients, especially Patient Scientists, as early as possible is a great way to set them up for success as a part of a clinical research study.⁵²⁻⁵⁵ Bringing patients in early will give them time to assimilate, learn how to be part of the process, and give them a sense of ownership of the research. Being involved early will improve the quality of patient input. Early involvement maximizes opportunities to gather and incorporate the patient perspective throughout the clinical trial process.

...and Often...

Each project will have a unique set of steps, tasks, information gaps, constraints, and potential outcomes that will inform decisions about when and how to involve patients. To maximize participation from your patients, integrate them into the clinical study team as much as possible. There are many ways to do so, including:

- Inviting them to the project kick-off meeting,
- Including them in all team conference calls and/or meetings,
- Copying them on all team communications, and
- Including them on smaller projects that will inform the shape of the final project.

...right 'til the very end.

Often forgotten in the process of research is sharing the results with all the study participants. Most often overlooked are the patients who participated in the research—not just as subjects but also as clinical research team members. There is an increasing appreciation that patients deserve to have access to their own clinical research data. At a minimum, patients who participated in a clinical research study should receive lay-friendly aggregated results of the study from the sponsor. Out of respect for their contributions, Patient Partners should be among the first with whom you share your clinical research results. Sharing clinical study results with patients increases transparency and trust between patients, sponsors, and the clinical research community.⁵⁶

Patient Scientists can be engaged further by:

- Giving them assignments, such as reviewing defined terms used in a protocol, to do on their own that they will then present to the team,
- Having them serve as liaisons to other patient groups working on the project and the patient community in general on behalf of the clinical research team,

⁵⁰ Irony, T., Ho, M., et al., “Incorporating Patient Preferences Into Medical Device Benefit-Risk Assessments.” *Statistics in Biopharmaceutical Research*. (2016)

⁵¹ Clinical Trials Transformation Initiative (CTTI), “Recommendations: Effective Engagement with Patient Groups Around Clinical Trials.” *Clinical Trials Transformation Initiative* (2015)

⁵² Patient-Centered Outcomes Research Initiative (PCORI), “Better Research through Engagement.” <https://www.pcori.org/sites/default/files/PCORI-Better-Research-Through-Engagement.pdf>. (2019)

⁵³ Patient-Centered Outcomes Research Initiative (PCORI), “Engagement Strategies, Challenges and Resources.” <https://www.pcori.org/sites/default/files/PCORI-Patient-Stakeholder-Engagement-Challenges-Strategies-Resources-Presentation-Slides-120517.pdf>. (2017)

⁵⁴ Clinical Trials Transformation Initiative (CTTI). CTTI Recommendations on Effective Engagement with Patient Groups Around Clinical Trials. <https://www.ctti-clinicaltrials.org/briefing-room/recommendations/ctti-recommendations-effective-engagement-patient-groups-around>. (2015).

⁵⁵ Patient-Centered Outcomes Research Institute (PCORI), “Highlights of PCORI-Funded Research Results.” <https://www.pcori.org/research-results-home>. (2019)

⁵⁶ Clinical Trials Transformation Initiative (CTTI), “Recommendations: Effective Engagement with Patient Groups Around Clinical Trials.” *Clinical Trials Transformation Initiative* (2015)



- Providing them the opportunity to speak publicly alongside the rest of the clinical research team about the clinical research study and the role of patients in the clinical study process (e.g., by including them at conferences, presentations, or with the media), and
- Including them as authors on study publications.

None of the above need take up excessive time and will likely enhance the clinical research study overall. Planning and scheduling of activities (such as regular team meetings) well in advance and setting up expectations for participation will help patients be successful contributors. Patients want to be involved. Once expectations and schedules are set, it is up to the patients to show up and be prepared.

No matter what the plan is to involve patients, keep these thoughts in mind:

- Clearly communicate patients' role in each task and setting. Some calls or meetings may be about understanding symptoms, in which patient input will be front and center. Some may be more focused on the science, in which the patients participate largely as listeners.
- Respect the time patients are donating. Committing significant time and energy to an endeavor in which you do not make a difference is frustrating for anybody, but particularly for someone living with the very condition that the endeavor was designed to address. Consider when planning any activity whether it may be perceived as “lip service” to the concept of patient involvement. Patients will happily dedicate hours to tedious meetings and calls if they believe that overall, they are making a difference.
- Patients want to be, and should be, heard. Giving them opportunities big and small to share their voices will be rewarding for everybody involved.
- Patients are more than just patients. If treated as one-dimensional, they will feel like outsiders and their commitment and enthusiasm may suffer. Appreciate that they have attributes beyond their condition that could be of value to the clinical research team. One patient might be a good writer, while another might be great at public speaking. Look for ways to include their skills for the benefit of the team.
- Try to be as flexible as possible. This may mean seeking input from patients at various points on how and when they want to be included or being willing to modify plans when appropriate. It may be that patients would prefer to be more or less involved than originally planned and making a change would be expensive or time-consuming. Clinical researchers should try to accommodate patients' requests regarding participation to the extent they are able (without doing harm to the clinical study or blowing the study budget). Sometimes a patient may suggest something that may not be possible. Rather than just ignoring or dismissing the suggestion, the researchers should educate the patients about why it is not possible and what alternatives they may be able to develop. Ultimately, it is the project sponsor's decision whether and how to incorporate patient perspectives.
- Respect the power of momentum and motivation. Momentum is real and it is powerful. Keep the wheels turning through effective communication. Avoid big gaps in involvement or interaction, which can sap interest levels. Consider a regularly scheduled update call or email (e.g., a 90-minute call twice a month for the whole team) to keep all team members, including the patients, informed. Determine what cadence is reasonable and set expectations for the group. Let your patients know how grateful you are for them and their contributions. Doing so is easy and will keep them excited about the project and their role.



What are the challenges to engaging patients?

Since involving patients actively in clinical research is a relatively new endeavor there will be a bit of a learning curve for all involved.

Researchers and sponsors may worry that engaging patients will be a burden or an overwhelming task that takes precious time and resources away from the research itself. For example, they may wonder if they will need to hire a special assistant just to manage the engagement of patients. It would be the rare study that would need a special staff position created to work with the patients. It is up to the clinical team to extend the opportunity of participation to patients and to define what that participation is and what it is not. It is up to the patients to seize the opportunity being extended to them, to take the time to understand what their role will be, and to show up!

There may be a disconnect with respect to the pace of the project. Researchers may find that the patients on the team do not fully understand the length and complexity of the research process. Patients, who are not familiar with the process and who are living with a serious medical condition, may understandably be somewhat frustrated with the pace of research. For patients, the research cannot go fast enough. They are looking for treatments and cures NOW. TODAY. Until they have a chance to better understand the research process, no matter how aggressive or “speedy” the research plan is, patients may push to speed things up. Providing and explaining a detailed timeline will help set expectations and allow both researchers and patients to ask questions and express their views.

Once patients understand the research study process, things may get worse before they get better. Patients may find study complexities daunting and confusing—so many steps, so many checkpoints, so many players. They may wonder if they really can offer valuable insights in such a multifaceted endeavor with so many stakeholders. They may wonder if they will be taken seriously. They may even wonder if there is room for them to influence the work. They may actually wonder if the project will ever end.

There undoubtedly will be hurdles along the way as research teams learn how best to engage patients and patients learn how best to contribute. Involving patients will not be problem-free, nor will you be able to plan for and mitigate all potential challenges. Acknowledging and addressing problems when they arise will not only help in the context of a particular research study but will also provide valuable learning for engaging patients in future research studies.

What benefits will result from engaging patients?

Despite the challenges that may occur, there is evidence of the value of including the patient voice in research. In brief, studies that actively engage patients can move faster, cost less, and produce more useful results.⁵⁷ Clinical researchers have a bigger impact by introducing higher quality research with greater relevance to those with the medical condition under study. And they have the satisfaction that comes from working directly with those they are trying to help. Regulators are taking note, as are payers. There is a growing sense that engaging patients should be part of best practices for clinical research. We encourage all clinical researchers and study sponsors to take the steps of engaging patients in their next study. We are certain they will not be sorry.

⁵⁷ Levitan, B., Getz, K., et al. “Assessing the Financial Value of Patient Engagement: A Quantitative Approach from CTTI’s Patient Groups and Clinical Trials Project.” (2018) *Therapeutic Innovation & Regulatory Science*, 52(2), 220–229.

A CHALLENGE TO THE FIELD: IDENTIFYING BEST PRACTICES FOR ENGAGING PATIENTS⁵⁸⁻⁶⁵

It is not obvious how to actively engage patients in clinical research to maximize the benefit of their input. And, currently, there are no recognized set of standards or best practices for engaging patients in clinical research. We believe that identifying some generally accepted best practices would be of value.

Throughout the published literature, thought pieces are emerging that describe what such best practices might include. However, much work is yet needed to be done. For example, while there are “guides for patients” when involved with clinical research, they mostly focus on being a subject in a clinical trial, not an active partner in the research.⁶⁶ Furthermore, though clinical trial sponsors (pharmaceutical and medical device companies, foundations, and the federal government, for example) and clinical researchers have developed their own protocols for engaging patients in their own research, there are no field-wide practices that all stakeholders can adopt or at least use as a starting point.

Important questions to be addressed in such field-wide best practices include the kinds of questions addressed in this paper—e.g., how to include patients and what to task them with. But these best practices should also address some larger issues outside the purview of this paper such as: should input from patients be a requirement for clinical trials used to support regulatory or reimbursement decisions? If so, should they be required in all trials or just some? How do we choose which ones must include patient input? How representative should the patients engaged in a clinical research study be of those affected by the research?^{67, 68} Should patients be paid for their engagement in clinical trials design and research?⁶⁹ Finally, who needs to “approve” or declare that “consensus” is reached on the best practices in order for them to be accepted and followed?

In closing, we challenge the field to purposely address setting best practice standards for engaging patients in clinical trials design and research, taking account of these types of questions.

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