

engagement strategies will be worth the investment; however, some seek tangible data to support this hypothesis. To provide this evidence, efforts to define and quantify the return on investment have been documented in the second phase of CTTI's patient group engagement project.

In 2016, CTTI presented a conceptual model to estimate the financial value of patient engagement based on expected net present value (ENPV), which integrates the key business drivers of cost, time, revenue, and risk into a summary metric for project strategy and portfolio decisions (Figure 1A) [2,58]. This helped the team outline what it would take to show the impact of a decrease-to-launch time and the total cost of the clinical trial enterprise as a framework for valuing patient engagement, as it has been difficult to make the fiscal value proposition. As a case example, CTTI assessed the impact of patient engagement on ENPV for a typical oncology development program entering phase 2 or phase 3. CTTI established that for a pre-phase 2 project, the

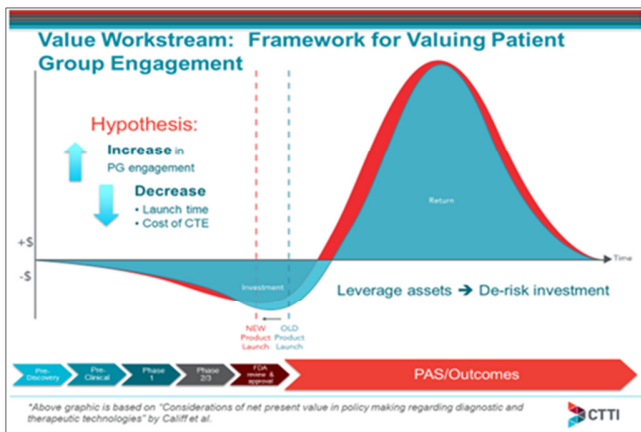


Figure 1A. Framework for valuing patient group engagement [58].

CONCLUSION

Patients and their advocates are taking more active roles in discovery and pre-clinical research, in the design and execution of clinical trials and in leading post-market evaluation activities. Patient groups are increasingly developing and strategically deploying assets to ensure the perspectives of their constituents are included in clinical research, leading to more rapid development of treatments that meet the needs of patients while decreasing risk and accelerating the therapy development process. Critical assessment of corporate culture and restructuring may be necessary to effectively engage patients early and often in the research and development continuum, as well as development of a structure to assess return on investment through emerging economic models.

DISCLOSURES

Selig: Nothing to report.

Patrick-Lake: Nothing to report.

cumulative impact of a patient engagement activity that avoids one protocol amendment and improves enrolment, adherence, and retention is an increase in net present value (NPV) of \$62MM (\$65MM for pre-phase 3) and an increase in ENPV of \$35MM (\$75MM for pre-phase 3). Compared with an investment of \$100,000 in patient engagement, the NPV and ENPV increases can exceed the investment by 500-fold. This ENPV increase is the equivalent of accelerating a pre-phase 2 product launch by 26 years (16 years for pre-phase 3). The work determined that risk-adjusted financial models can assess the impact of patient engagement by using a combination of empirical data and subjective parameter estimates, which correlate patient engagement activities with the potential to avoid protocol amendments and/or improve enrollment, adherence, and retention (Figure 1B) [2]. The third phase of CTTI's work is focused on developing tools to help sponsors identify high-value patient engagement activities for investment.

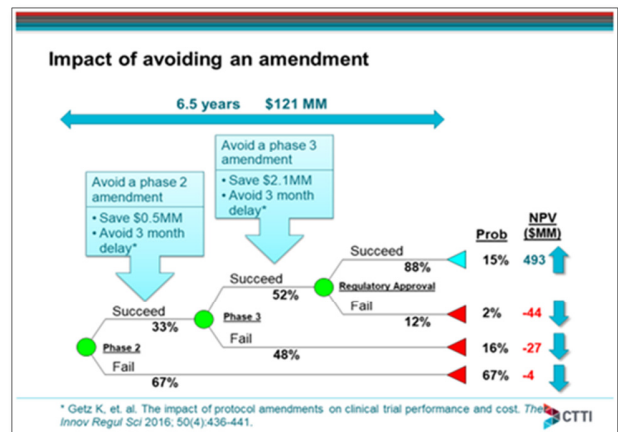


Figure 1B. Impact of avoiding a protocol amendment [2].

REFERENCES

1. Anderson M, McCleary KK (2015) From passengers to co-pilots: Patient roles expand. *Sci Transl Med* 7: 291.
2. Levitan B, Getz K, Eisenstein EL, Goldberg M, Harker M, et al. (2017) Assessing the financial value of patient engagement: A quantitative approach from CTTI's patient groups and clinical trials project. *Ther Innov Regul Sci* 52: 220-229.
3. <http://www.lung.org/about-us/mission-impact-and-history/our-history.html?referrer=https://www.google.com/>
4. <https://www.cancer.org/about-us/who-we-are/our-history.html>
5. https://www.heart.org/HEARTORG/General/About-Us--American-Heart-Association_UCM_305422_SubHomePage.jsp
6. <http://www.jdrf.org/about/>

7. Manganiello M, Anderson M (2011) Back to basics: HIV/AIDS Advocacy as a model for catalyzing change. Available at: <http://www.fastercures.org/assets/Uploads/PDF/Back2BasicsFinal.pdf>
8. <https://ww5.komen.org/AboutUs/OurWork.html>
9. <http://www.breastcancerdeadline2020.org/about-nbcc/>
10. <http://cdmrp.army.mil/bcrp/>
11. <https://www.pcori.org/about-us>
12. Osuch JR, Silk K, Price C, Barlow J, Miller K, et al. (2012) Historical perspective on breast cancer activism in the United States: From education and support to partnership in scientific research. *J Womens Health (Larchmont)* 21: 355-362.
13. <https://fightcolorectalcaner.org/advocacy/research-advocacy/>
14. <https://ww5.komen.org/GetInvolved/Participate/BecomeanAdvocate/BecomeanAdvocateinScience.html>
15. <http://cdmrp.army.mil/aboutus>
16. <https://www.healthra.org/membership/member-profiles>
17. <https://www.pcf.org/c/ceo-message/>
18. <https://www.michaeljfox.org/foundation/promise.html?navid=our-promise>
19. <https://www.centerwatch.com/news-online/2015/08/17/three-questions-wendy-k-d-selig-wscollaborative/>
20. <https://www.centerwatch.com/news-online/2015/08/17/three-questions-wendy-k-d-selig-wscollaborative/>
21. <http://train.fastercures.org/about/what-is-venture-philanthropy/>
22. Robinson R (2016) Patients and patient organizations power rare disease therapies. *PharmaVoice*. Available at: <http://www.pharmavoice.com/article/2016-02-rare-disease-therapies>
23. <https://www.fda.gov/ForIndustry/DevelopingProductsforRareDiseasesConditions/OOPDNewsArchive/ucm292294.htm>
24. <http://pcornet.org/patient-powered-research-networks/pprn15-parent-project-muscular-dystrophy/>
25. <http://www.cff.org/Research/Researcher-Resources/Patient-Registry/>
26. <http://www.curefa.org/patient-registry>
27. <https://pdsa.org/join-the-community/registry.html>
28. <http://www.fastercures.org/assets/Uploads/PDF/Patient-Registries.pdf>
29. FDA (2017) Awards six grants for natural history studies in rare diseases. U.S Food & Drug Administration. Available at: <https://www.fda.gov/NewsEvents/Newsroom/PressAnnouncements/ucm579375.htm>
30. (2015) CTTI recommendations: Effective engagement with patient groups around clinical trials. *Clinical Trials Transformation Initiative*. Available at: <https://www.ctti-clinicaltrials.org/files/pgctrecs.pdf>
31. <https://www.themmrp.org/research-partners/mmrp-learning-network/translational-network/>
32. Peay H, Fischer R, Furlong P, Bridges JFP, Hollin I (2018) PPMD's patient preference study about pulmonary outcomes - What we learned and why it matters. *Parent Project Muscular Dystrophy*. Available at: http://www.parentprojectmd.org/site/DocServer/Santhera_Community_Report_v03.pdf?docID=16886
33. <https://www.lungevity.org/research/patient-focused-research-center-patient-force/deriving-patient-preferences-project>
34. <https://www.asco.org/advocacy-policy/asco-in-action/asco-and-friends-cancer-research-release-comprehensive>
35. <https://www.pancan.org/research/precision-promise/>
36. <http://news.cancerconnect.com/lls-launches-groundbreaking-precision-medicine-approach-treat-alm/>
37. <http://nbdabiomarkers.org/gbm-agile>
38. Woodcock J, LaVange LM (2017) Master protocols to study multiple therapies, multiple diseases or both. *N Engl J Med* 377: 62-70.
39. <https://www.cff.org/About-Us/About-the-Cystic-Fibrosis-Foundation/CF-Foundation-Venture-Philanthropy-Model/>
40. <http://www.jdrf.org/about/t1dfund/>
41. <https://www.lls.org/therapy-acceleration-program>
42. (2016) Key considerations in developing and integrating patient perspectives in drug development: Examination of the Duchenne case study. *Parent Project Muscular Dystrophy*. Available at: https://www.bio.org/sites/default/files/BIO_PPMD_whitpaper_web.pdf
43. Barron D (2017) Patient engagement: Great expectations. *Eye for Pharma, Barcelona*. Available at: <http://social.eyeforpharma.com/commercial/patient-engagement-great-expectations>

44. Schneider RF, Pankevich D (2018) Patient First” Beyond a Slogan, a Drive for Full Inclusion. Huffington Post. Available at: https://www.huffingtonpost.com/entry/patients-first-beyond-a-slogan-a-drive-for-full-inclusion_us_59c02882e4b087fdf5075781
45. Mack J (2015) The Debut of the Chief Patient Officer: Is it just a passing fad or will it transforms. Pharma Marketing News. Available at: <http://www.pharmamkting.com/news/pmnews1403-article01.pdf>
46. <http://www.celgene.com/partnerships/advocacy-partnerships/>
47. https://get.knect365.com/clinical-trials-tech-transformation/patient-rd-scrip/?utm_source=clininnovation&utm_medium=blog&utm_campaign=clinicalcontent&utm_content=scrip_patient_rd_120118&_ga=2.140930034.1560957657.1516110627-1747083652.1516110627
48. <http://www.fastercures.org/programs/r-and-d-policy/ufas/>
49. Pukita V (2018) FDA’s Patient-Focused Drug Development. U.S. Food and Drug Administration. Available at: <https://www.fda.gov/downloads/drugs/newsevents/ucm493616.pdf>
50. U.S. Food and Drug Administration (2017) Plan for Issuance of Patient-Focused Drug Development Guidance. Available at: <https://www.fda.gov/downloads/ForIndustry/UserFees/PrescriptionDrugUserFee/UCM563618.pdf>
51. Kuehn CM (2018) Patient Experience Data in US Food and Drug Administration (FDA) Regulatory Decision Making: A Policy Process Perspective. *Ther Innov Regul Sci* 52: 661-668.
52. National Health Council (2015) Dialogue/advancing meaningful patient engagement in research, development and review of drugs. Available at: <http://www.nationalhealthcouncil.org/sites/default/files/PatientEngagement-WhitePaper.pdf>
53. <http://phrma-docs.phrma.org/sites/default/files/pdf/patient-focused-drug-development.pdf>
54. Peretto EM, Oehrlein EM (2015) Assessing meaningful patient engagement in drug development: A definition, framework and rubric. CERSI University of Maryland.
55. Bloom D, Beetsch J, Harker M, Hersterlee S, Moreira P, et al. (2012) The rules of engagement: CTTI recommendations for successful collaborations between sponsors and patient groups around clinical trials. *Ther Innov Regul Sci* 52: 206-213.
56. Leonard K (2012) The blockbuster drug of the century: An engaged patient. *Health Standards*. Available at: <http://healthstandards.com/blog/2012/08/28/drug-of-the-century/>
57. Carsten B (2017) making real progress on infusing the patient voice into oncology clinical development. *Medium*. Available at: <https://medium.com/@bayerus/making-real-progress-on-infusing-the-patient-voice-into-oncology-clinical-development-18b3cf6f7fe4>
58. Califf RM, Raisel EB, Schulman KA (2008) Considerations of net present value in policy making regarding diagnostic and therapeutic technologies. *Am Heart J* 156: 879-885.