



Measurement of Impact

THE *FASTERCURES* CONSORTIA-PEDIA REPORT

About the *FasterCures* Consortia-pedia project:

FasterCures initiated the Consortia-pedia project to better understand the breadth and scope of approaches that a wide range of consortia have adopted to bring together non-traditional partners with a shared R&D goal. Since 2012, our analysis of more than 350 biomedical research consortia has been aimed to better understand how different stakeholders are using this model of partnership to address shared unmet needs.

To better understand consortia models, *FasterCures* analyzed 21 efforts that represent the diversity of models used to bring together non-traditional partners to accelerate biomedical research. We present our analysis under seven partnership components.

1. Governance
2. Financing
3. Human Capital
4. Intellectual Property
5. Data Sharing
6. Patient Participation
7. Measurement of Impact

Each component is a chapter in the Consortia-pedia report and can be downloaded at:
www.fastercures.org/consortiapedia.

UPDATED May 2014

KEY POINTS – IMPACT:

- **Understand the expectations** of each participant and sponsor within a consortium.
- **Establish metrics** that align with the mission statement and can be measured periodically throughout the course of the collaboration.
- **Design a mid-term assessment** that communicates progress to individual stakeholders and can also be used operationally to identify opportunities for optimizing collaboration.
- **Communicate your findings** in a manner that is specific to the audience of interest.

The cross-sector retraction of resources has changed the biomedical research ecosystem from one of independent silos toward one defined by increased collaboration. As highlighted throughout the Consortia-pedia project, *FasterCures* has seen a large growth in the number of multi-sector biomedical research consortia emerging in the past decade. But there is limited real estate within the research landscape because all depend on the same pool of financial resources, intellectual capital, and patient populations. The individual investment varies – from industry’s contribution of finances and in-kind resources, to the patient who contributes his or her time, biospecimens, and hope – and all seek to maximize the return from their participation.

This becomes a challenge to the leadership of any consortium as they are not only faced with coordinating the pursuit of an ambitious scientific agenda, but must also manage a collaboration that provides the maximum value to all stakeholders. Contributing to the challenge is a landscape that does not appear differentiated – there are a large number of consortia that appear to have the same mission and purpose. This ends up not being the case; in *FasterCures’* analysis of more than 350 biomedical research consortia, the majority had different research strategies and work-streams, with very few having research agendas that overlapped.

The confusing consortium landscape is forcing many consortium participants to limit their participation to collaborations that have the greatest chance of providing a benefit to their organization. To help mitigate their risk, many participants are only providing a limited commitment that will only be renewed after assessing whether the consortium is on-course to realizing its value proposition. Participants are increasingly asking for evidence at earlier stages of a consortium’s expected lifetime. Many consortia are struggling to find ways to provide these details, and *FasterCures* found that very few consortia implement some level of value assessment. There are several reasons, but one challenge that emerged from our discussions with consortium leaders is that expectations of output and value are dependent on the unique interests of each specific stakeholder, and very few of these are well-defined at the operational level.

Assessing properly defined metrics throughout a consortium’s lifespan has several potential uses, including:

- Providing evidence to consortium sponsors and participants that their contributions of time and resources are being used efficiently and effectively
- Identifying opportunities to optimize operational efficiency by the consortium leadership and managers
- Providing a level of transparency and accountability to consortium participants and the broader public
- Providing evidence for follow-on funding to expand or continue the research projects

The pharmaceutical industry is one sector that is becoming increasingly cautious in its commitment to new consortia. Some of the larger companies that we interviewed were involved in more than 50 consortia, and others were involved in more than 100 consortia. This excitement to collaborate also brings the unintended consequence of creating an atmosphere of “consortium fatigue,” as very few interviewees were able to describe how the products of these consortia would integrate within their internal operations. To help with the internal management of these collaborations, increasing numbers of companies employ “alliance managers” who serve as liaisons between the company's leadership and all of their external partnerships, including consortia. These teams are responsible for continually monitoring the value that each partnership provides to their company's business strategy, basing their assessments on data provided by the consortium and surveys of employees

directly involved in the partnership activities. A number of consortium sponsors and participants, ranging from industry to government, are now dedicating one of their staff members to consortium management activities.

In response to these pressures, some consortia are making strategic efforts to demonstrate and communicate the value that their partnership model adds to the biomedical research ecosystem. Many of these groups are also customizing their evidence of value proposition toward a specific stakeholder's interests.

There are some commonalities in their approaches:

- Impact or value statements look beyond the consortium leadership's expectations and are customized to the unique interests of sponsors, participants, and beneficiaries.
- The anticipated impact of the partnership is clearly described as part of a consortium's mission statement.
- The mission statement is broken down into research objectives that provide a pathway to the consortium's intended impact.
- Metrics that represent the anticipated impact of the collaboration are defined at the onset and represent all of the research activities, not just the achievements of a single project.
- Metrics are periodically measured and evaluated to track the progress toward consortium goals throughout its lifespan and reflect the mission, level-of-risk, and timeline.

Impact is phase-specific, not project-specific

A consortium's timeline should include periods of assessment that aim to measure the effectiveness of its collaboration model. If successfully described, these data points can be used to attract additional sponsors and participants. Alternatively, these measurements can also be used to identify inefficiencies at the earlier stages and determine if there is room for improvement. These metrics reflect all projects and are different from the technical/scientific milestones used for a specific project.

Measuring CIMIT's impact

The Center for Integration of Medicine and Innovative Technology (CIMIT) initiated a comprehensive Clinical Impact Study to examine the accumulation of publications, project reports, grant submissions, and investigator experiences from all projects. The goal of this study was to identify areas that the CIMIT model could improve.

The results of the study indicated:

- Projects completed in clusters, facilitated by networked peers across sectors, are more effective than projects that have expertise in only one area or technology.
- Seed grants of \$100,000 to \$300,000 is the financial amount that is the most cost-effective in creating impact with the greatest leverage effect (approximately 11x return).
- Time alone did not correlate with success – projects could be prioritized to look for early indications of potential success.
- Targeted and skilled project facilitation is a powerful factor at any stage of a project's execution.

CIMIT uses CoLab, a cloud-based platform that enables a team, group, or network to manage its technology development. The implications of the Clinical Impact Study on CIMIT's future activities include: continued emphasis on pre- and post-award facilitation; discontinuation of larger grant options in favor of seed grants of \$100,000 or less (with additional "accelerator" funding of \$200,000 available for intensive facilitation to speed projects showing great promise of a commercial exit); and increased focus on newer investigators.

As one example, the Innovative Medicines Initiative (IMI), a 10-year initiative with more than 40 programs, launched in 2006. It distributes its assessment milestones into three cross-IMI stages that approximately reflect:

- short-term impacts (approximately two to three years after launch);
- mid-term impacts (after four to five years); and
- long-term impacts (after seven to eight years).

Periodic impact assessments help refine the collaboration model

IMI planned its impact measurements according to the timeframes described earlier to allow time to improve its model and justify the continued public and private support after its 10-year proof-of-concept.

The following activities looked across all of its research efforts:

- | | |
|------------|--|
| 2007: | A panel of outside experts produced a report forecasting the anticipated economic and societal effects. Using interviews of experts and data/statistics from public resources such as Eurostat and the Organisation for Economic Co-operation and Development, these experts predicted that IMI has the potential for increasing the competitiveness of the European continent for drug discovery, as well as promote jobs and economic growth. |
| 2011: | A panel of independent experts were convened by the European Commission (EC) to evaluate the IMI's progress. In addition to its positive comments, this panel also identified the need to improve the methods used to demonstrate the initiative's impact. IMI's Scientific Committee began self-assessment of all projects. The final report made recommendations to update IMI's research strategy in accordance with the landscape of the pharmaceutical sector at that time.
IMI's Scientific Committee began surveys and workshops aimed at assessing the level of impact that IMI has made on its various stakeholders. |
| 2012-2013: | IMI began annual bibliometric analyses of its ongoing projects to evaluate its model of collaboration and quality of research, in response to the recommendations made by the EC's panel. |

As another example, the Biomarkers Consortium used a retrospective analysis at the end of its first project as an opportunity to test and learn from its collaboration model, gaining lessons that have guided its strategies for 11 ongoing projects. Based on its operational assessment of the Adiponectin Project, its new efforts use modified procedures for project management, utilize different collaboration tools to convene the participants, address data sharing principles and standards earlier in the process, and also have more realistic expectations for the use of existing data. This is an example of how the retrospective analysis of impact can be used to optimize the processes of future consortia.

Other groups have also modeled a staged approach to consortium development by incorporating periods of proof-of-principle assessment. For example, TransCelerate BioPharma has a strategic plan to make progress in incremental phases that are incorporated with objective value assessments, with the launch of the most ambitious projects occurring after its collaboration model has been refined and optimized.

Communicating impact to a specific audience

While there are some impact metrics that are of interest to all stakeholders, impact summaries should also be tailored to specific audiences, which not only include funders and sponsors, but also individual participants who want to feel that their time is well-spent, those studying collaboration models that have and have not worked, and society who should ultimately benefit from these consortia. This obviously increases the complexity of these assessments, but audience-tailored impact measurements have been shown to be important tools that help gain and strengthen support in an era of fiscal conservatism and consortia fatigue. Table 1 is a simplified list of various stakeholders with a general description of impacts that may be of interest.

Table 1: Expectations of different consortia audiences

Sector	Examples of expectations
Public	<ul style="list-style-type: none"> • Increasing the flow of new medical products reaching patients • Stimulating and sustaining economic vitality • Maintaining productive communities and ensuring tax revenue by supporting local industry • Addressing challenges in translational pathways by spreading the risk of creating broad-based tools and methods • Increasing global competitiveness in the biomedical sector • Supporting and de-risking the pursuit of new research opportunities, reducing redundancies • Leveraging private funding to maximize taxpayer dollars • Engaging in scientific discourse that can help inform regulatory and reimbursement policies in a forum that is not part of a formal regulatory process • Advancing the general health of society by lowering the costs and accelerating the speed of drug discovery
Private	<ul style="list-style-type: none"> • Sharing the risk of creating broad-based tools and methods to increase the speed and/or lower the costs of their own development processes • Cost-sharing the risk for entering new research opportunities, access to academic innovation, and resources • Getting access to patient populations and expertise on specific diseases • Sharing and combining data to increase statistical power, accelerated validation through consensus • Developing and validating standards that are recognized by regulatory authorities and can accelerate R&D timelines and product review • Developing partnerships that potentially lead to additional resources from those inside and outside of their sector (academics, patient groups, government)
Patients	<ul style="list-style-type: none"> • Providing opportunities to engage patient populations in biomedical research • Coordinating the expertise and resources in academia and the private sector around their disease of interest • Leveraging funding from the private and public sectors • Enabling data-sharing and collaboration among researchers
Academic	<ul style="list-style-type: none"> • Translating basic research findings to an application that meets an identified need • Accessing alternative non-government sources of funding • Accessing technology development resources from industry, such as compound libraries, datasets, or biostatistics expertise • Participating in new collaborative efforts that place them at the forefront of state-of-science • Developing partnerships outside of the consortium that could lead to additional resources • Improving their clinical care model

Public sector

Government – at the federal, state, or regional level - initiates or participates in multi-stakeholder consortia for a variety of reasons that depend on the mission of their agency and authority. The government audience is not limited to those agencies that have a biomedical research mission; many consortia also have sponsoring agencies that are interested in economic growth and job and company creation. As general trustees of taxpayer interests, government-initiated consortia need to have impact summaries that are tailored to these stakeholders, as their participation and support need to be justified from the public’s perspective. Transparency in both the mission, purpose, and operations is critical.

Public-sector consortia often share the interests of nonprofit foundations that have similar mission statements. For example, the Flinn Foundation is the primary backer of the Arizona Biosciences Roadmap, which has the goal of making the state of Arizona a leader in biomedical research through industry growth and job creation. The foundation’s support was crucial in launching the Critical Path Institute. Academic-sector and nonprofit patient organizations are treated as separate audiences within the Consortia-pedia project, even though most consider these groups as part of the public sector.

Private sector

As the group that is largely responsible for translating biomedical research into products that can be used in the clinic, the private sector includes large industrial companies and small-to-medium sized businesses that have a focus on making a profit from the production of medical tools. The groups typically participate in consortia because they identified an operational inefficiency that could be addressed by sharing expertise, laboratory resources, and funding.

Academic sector (including nonprofit research institutions)

This nonprofit sector primarily conducts the early-stage research that leads to innovative medical products. At the other end of the spectrum from basic research, many of these groups are also integrated with patient care systems and serve as a resource for clinical trials or for testing new models of clinical care. These groups typically participate in consortia to access the resources from other sectors – such as industry resources and expertise or access to specific patient populations. Consortia-based collaborations also offer an opportunity to co-author publications destined for peer-reviewed literature.

Patient organizations

These nonprofit organizations are focused on increasing the quality of life for a specific patient population. These groups are less interested in advancing fundamental science unless it has direct applications to advancing innovations in their disease of interest. In addition to providing disease expertise and access to a patient base for clinical trials, many of these groups have venture philanthropic activities to finance R&D activities that further advance their interests.

Assessing output of collaborations

Most consortia have the side goal of demonstrating that a specific model of multi-sector collaboration can improve efficiencies in biomedical research, with the hope that the collaborations will be sustained after the consortium has officially ended. The metrics used to measure output should be meaningful to the audience that is interested in the consortium. Metrics such as an improvement in the time or cost of developing medical products are hard to define mid-term and are typically part of a retrospective analysis after the completion of the collaboration. For other impact assessments, there are no systematic methods that can be used to compare across consortia, with many organizations relying on those used by academic researchers to assess the impact of interdisciplinary collaborations, such as number of patents, number of publications and the quality of journals, or number of citations. There is a critical need to create definitions for these metrics, with methods for their assessment.

For example, one of IMI's missions is to have a "structuring" effect in Europe by serving as a neutral ground for collaboration. To address this expectation, IMI developed a "collaborative index" that is based on an evaluation of the papers emerging from all of its research teams. Its 2013 Bibliometric Report evaluated the 320 publications from the various IMI efforts since the launch of the initiative and reported the following statistics:

- 73.1 percent of the publications were co-authored by more than one IMI-supported researcher
- 40 percent of the publications were the results of cross-sector collaborations
- 25 percent of the publications were reported results of cross-project collaborations
- The average citation impact (1.34) for IMI publications is higher than the world-wide (1.0) and European average (1.14)
- Collaborations of researchers with patient organizations have produced stronger publications

The Alzheimer's Disease Neuroimaging Initiative (ADNI) also used citation indices to demonstrate how the sharing of clinical trial data among researchers can improve academic and industry research on Alzheimer's disease. This consortium actively tracks the publications that use ADNI data¹ and published a comprehensive review of these publications in a 2012 edition of the journal *Alzheimer's and Dementia*.¹ In addition to bibliometric citations, ADNI also actively tracks data requests and reports the attributes of the various data requestors by location and sector. An additional metric of success was the replication of the ADNI collaboration model in Europe, Asia, and Australia, leading to an exponential growth in available data for the Alzheimer's disease research community.

¹ Weiner, MW et al., The Alzheimer's Disease Neuroimaging Initiative: a review of papers published since its inception, *Alzheimer's and Dementia*, 8 (2012), S1-S68.

Assessing outcomes of collaboration

The outcomes of consortia differ from the output, in that the broader impact of the consortium is the metric that is being used. Typically, the output should lead to an outcome that provides evidence that the consortium has made an impact. As with the output, the interest in a specific outcome depends on the audience.

Accelerating development of cures

Many consortia that are driven by patient foundations or government have a mission to accelerate the development of a cure for a specific disease. The Multiple Myeloma Research Foundation (MMRF) has also quantified its impact in accelerating the clinical trials run through its Multiple Myeloma Research Consortium (MMRC). According to its Web site, MMRF reports that MMRC trials are opened 60 percent faster and complete enrollment 12 percent faster compared to similar clinical trials. It also states that its ability to establish 45 clinical trials on more than 20 candidate therapeutics has helped to catalyze the U.S. Food and Drug Administration (FDA) approval of six treatments.

The Collaborative Chronic Care Network (C3N) aims to improve the clinical care of children with chronic illnesses using a learning-based network that relies on data input from patients, clinicians, and researchers. The system is being tested within a large care network known as ImproveCareNow. While it is still in the early stages of implementation, this network uses several clinical metrics that can be used to track the impact of C3N's platform technologies. For example, the remission rates of pediatric patients with irritable bowel disease in the network improved from 50 percent to 75 percent over four years, and this metric will be used to see if C3N can further increase its performance.

Creating broadly used tools and methods

Some consortia focus on catalyzing the development of new tools and methods that advance biomedical research for all stakeholders. These new tools can include biomarkers and assays, diagnostics, methods for clinical trials, or clinical decision technologies. An accelerated timeframe is the metric that is often used, and others may be interested in improved efficiencies such as reduced patient numbers for clinical trials. For example, the ADNI review described earlier evaluated the initiative's impact on creating new research opportunities and clinical care methods for Alzheimer's disease. The authors determined that ADNI data catalyzed the creation of standardized clinical diagnostics, imaging methods and biomarker research opportunities, methods for improved and efficient clinical trials, knowledge that can be used to provide a greater understanding of the aging process, and methods for data sharing.

The Critical Path Initiative's Coalition Against Major Diseases created a simulation tool that can be used to design the clinical trials of treatments against mild and moderate Alzheimer's disease. The potential outcome of this tool could be faster clinical trials with smaller patient populations, but it would only be a scientific exercise if the regulatory bodies that would evaluate the results from these trials didn't validate the utility of the tool. More importantly, the tool would see little adoption without this certification. Fortunately for this consortium, both the FDA and the European Medicines Agency issued notices that recognize the validity of this drug development tool. The outcome of having this tool accessible to the entire research community is potentially simpler clinical trials and regulatory review processes for any new therapeutic candidates.

Another similar impact was demonstrated by the Biomarkers Consortium's Adiponectin Project, which addressed the need to validate a biomarker that can stratify a subset of Type 2 diabetes patients who respond to a certain type of drug. Through this consortium, four pharmaceutical companies each contributed pooled and blinded clinical trial data that were used to validate the performance of this biomarker. In terms of direct impact, these results are expected to accelerate the development of drugs that use this diagnostic biomarker since the evidence and results were broadly shared, including with the FDA, who may use these data in the evaluation of any new drug candidates.

Economic growth

Some government agencies and nonprofit organizations view biomedical research as an economic driver and want to leverage collaboration to stimulate and sustain this type of growth. The approaches for demonstrating economic growth can include job creation, creating/maintaining a knowledge base, leveraging taxpayer dollars with private funding, and supporting an innovation ecosystem for company creation. One metric that is often reported is the leverage effect, where the pooling of funds and resources by multiple groups results in increased buying power for any individual participant.

Impact measurements for economic growth are based on quantitative analysis, and there are several ways to summarize the data. The greatest opportunity to evaluate the economic effects of collaboration is after the consortium's lifespan as part of a retrospective assessment. For example, an economic report published by Battelle and United for Medical Research showed how the initial investment of \$3.8 billion into the Human Genome Project resulted in an economic output of \$796 billion, personal income exceeding \$244 billion, and the creation of 310,000 jobs between 1988 and 2010 – an economic generation of \$141 for every \$1 invested by the federal government. The report also stated that the industry stimulated by this collaboration had returned \$3.7 billion in federal taxes and \$2.3 billion in state and local taxes to the U.S. economy in just one year (2010).

Such long-term data may not be realistic for consortia that are at the earlier stages of maturity, and there are other methods for summarizing mid-term impact. For example, the Quebec Consortium for Drug Discovery's (CQDM) mission statement includes the stimulation of the Quebec bioscience economy, and it does this by leveraging private and public funding to sponsor more than 30 projects. As a moderately mature consortium that has projects with a definitive end-date that occur during the lifespan of its consortium, CQDM has a sufficient level of evidence to conduct a retrospective mid-term impact analysis. An impact assessment has shown that 32 of its research projects have resulted in an additional \$14.5 million in the provincial bioscience economy and the creation of more than 250 jobs, with an average leverage effect across all of their programs of \$20 for every \$1 contributed. In addition, this consortium also tracks the progress of their projects afterwards to document follow-on funding from other sources, as a metric that demonstrates the consortium's ability to de-risk innovative concepts.

Since CQDM's activities are spread over multiple independent efforts, CQDM also analyzes its contribution to the regional economy. As one example, the Quebec-based company Medicago received \$1.8 million from CQDM at its early stages to develop a new vaccine production platform called VLPEXpress. Both the financial support and CQDM's model of industry mentorship helped Medicago demonstrate the robustness of its technology and resulted in an additional \$10 million in venture round funding, \$21 million from the U.S. Department of Defense, and \$48 million from partnerships with other pharmaceutical companies. The company is now publicly traded. In 2013, Mitsubishi Tanabe Pharma took a majority 54 percent stake in Medicago worth up to \$172 million. In addition to the impact on job creation, Medicago developed a pilot production facility in Quebec, further increasing the economic impact of the original CQDM investment.

Other groups, such as the IMI, have an objective to improve the European Union (EU) economy by leveraging non-public funds to "stimulate European competitiveness within the biopharmaceutical sector." As part of its assessment, IMI conducted a survey of its participants, and 35 percent of the respondents reported that IMI funding facilitated access to other funds. IMI estimated that its funding has created more than 1,500 jobs in the EU. This represents one job created for every €200,000 of public funding, compared to prior EU-level programs that created one job for every €400,000 of public funding.



For more information and the latest updates on the *FasterCures* Consortia-pedia, visit www.fastercures.org.

ⁱ <http://www.adni-info.org/Scientists/ADNIScientistsHome/ADNIPublications.aspx>, accessed on 12/31/2013