Understanding the Role of Patient Advocacy Organizations in Funding Medical Product Research and Development

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Executive Summary

Background

Patient advocacy organizations (PAOs) play a key role in medical product development. Many PAOs raise money to fund medical product development activities – such as basic science research, preclinical research, and clinical trials - for their condition of focus, whereas others fund or develop resources such as biobanks or patient registries to support the research community. PAOs fund medical product development by providing financial support to academic and medical institutions and life sciences companies, or by conducting research in house. However, little is known about the PAOs that are engaged in funding these activities.

To assess PAOs' engagement in funding medical product development, Mathematica conducted a study to learn about the types of medical product development activities that PAOs fund, the amount of funding they dedicate to these activities, and their approaches to funding such activities. Mathematica developed a list of 1,555 PAOs that operate in the United States, starting with the 1,215 PAOs in the Kaiser Health News Pre$cription for Power dataset and supplementing it with searches of additional data sources. For each PAO on the list, Mathematica reviewed its website and most recent Tax Form 990 (generally from 2022 or 2023) to determine whether the PAO funded medical product development activities and if so, to document information about the PAO's engagement in medical product development.

Findings[1]

Of the 1,555 PAOs on the list, 585 PAOs (38 percent) reported funding medical product development activities. We refer to the 585 organizations that fund medical product development as *PAO funders*. Key findings from the study include:

- Sixty percent of PAO funders (350 PAOs) have an annual revenue greater than $1 million. PAOs with the highest revenue are generally older and more established than those with lower revenue. However, approximately one-quarter of PAO funders had less than $500,000 per year in annual revenue.
- Basic science research, such as natural history studies, was the most common activity that PAOs reported funding (79 percent of PAO funders). The second most common type of activity reported by PAOs was preclinical research, such as *in vivo* or *in vitro* studies and high throughput screening studies (52 percent of PAO funders). Fewer PAOs reported funding patient registries or biobanks (34 percent of PAO funders) or clinical trials (35 percent of PAO funders). PAO funders with higher revenue were more likely than those with lower revenue to report funding medical product development activities—especially clinical trials.

[1] The counts presented in this report, such as the number of PAOs that fund various medical product development activities, should be interpreted as a minimum rather than the true number of PAOs engaging in this work. Because the study methods rely on publicly-available information on PAOs' websites and tax forms, and PAOs generally did not report this information in a standardized way, there are likely additional PAOs that fund medical product development activities that were not captured in the dataset.

- The amount of funding PAOs reported providing for medical product development activities varied widely, ranging from $2,000 per year to over $300,000,000 annually. Twenty-four percent of PAO funders provided less than $100,000 per year on average; 48 percent provided $100,000 to $999,999; and 29 percent provided more than $1 million. In general, PAOs with higher revenues also dedicated more funding to medical product development activities.
- Ninety-two percent of PAO funders reported funding academic or medical institutions, typically through competitive grant processes. Fewer PAO funders (18 percent) reported funding life sciences companies directly. PAOs that reported funding life sciences companies have a higher average revenue than those that do not fund life sciences companies. This suggests that smaller and newer PAOs may face barriers to establishing funding arrangements with life sciences companies.
- Another less common approach to funding medical product development activities was to conduct research and development in house using PAO staff. This included PAOs that reported using PAO staff and resources to conduct their own early-stage research (14 percent of PAO funders); conducting clinical trials at the PAOs' service delivery sites (1 percent of PAO funders); operating primarily as nonprofit life sciences organizations with their own research staff and labs (2 percent of PAO funders), and using substantial financial resources to support other in-house medical product development activities (2 percent of PAO funders).

Discussion

The study findings highlight the extensive involvement of PAOs in advancing medical product development. While PAOs of all sizes reported funding medical product development activities, PAOs with fewer resources might benefit from additional support to overcome challenges in doing so, such as peer learning opportunities, new formal channels for establishing partnerships with academic researchers and life sciences companies, and opportunities to grow their revenue (and thus, dedicate more funding to medical product development). Additionally, the study highlighted the lack of publicly available data on funding provided by PAOs to life sciences companies. This lack of transparency further poses a challenge to PAOs that are interested in funding life sciences partners, making it harder for them to identify other PAOs that have done this work and to learn from them. Given the successes PAOs have experienced in advancing medical product development by providing financial support for these activities, it will be important to think about ways to engage PAOs in this work and help them sustain their efforts—especially PAOs that are relatively small and less well established.

I. Introduction

Patient advocacy organizations (PAOs) play a key role in medical product development. Many PAOs raise money to fund medical product development activities (defined in Exhibit 1) for their condition of focus, whereas others fund or develop resources such as biobanks or patient registries to support the research community. These efforts are intended, in part, to address unmet needs for specific health conditions by closing gaps in funding for drug and medical device research (Stevens 2019).

Many PAOs fund research for their health conditions of focus. According to a 2012 survey of 201 PAOs, 60 percent provided funding for clinical research, and 45 percent had supported a patient registry or biobank in the past two years (Landy et al. 2012). More recently, a 2023 survey of 225 rare disease PAOs showed that 79 percent engaged in research activities, and of these, about half initiated and funded the research (Patterson et al. 2023). As of 2021, an estimated 159 U.S.-based PAOs run patient registries (IQVIA 2023).

To support medical product development activities, PAOs mainly rely on funds from charitable contributions, including from life sciences companies. Contributions from individual donors and corporations (including life sciences companies) account for roughly 80 percent of PAOs' total revenue, whereas program services account for only 12 percent (IQVIA 2023). More than half of PAOs have received funding from life sciences companies (Kaiser Family Foundation Health News 2019), and a survey of PAOs in the United States revealed that 12 percent received more than half of their funding from life sciences companies (Rose et al. 2017). Some experts have expressed concerns that donations from life sciences companies to PAOs could incentivize PAOs to prioritize the interests of life sciences companies over those of the patients they represent, whereas others see these donations as a helpful way for PAOs to bolster resources for strategic initiatives, including research for life-saving medical products.

Exhibit 1. Study definitions

We used the following definitions for the study:

Patient advocacy organizations (PAOs) are 501(c)(3) nonprofit groups devoted to assisting patients with a specific disease, disability, or condition beyond simply providing services or care. This assistance includes funding or conducting research; raising awareness; and lobbying to support or oppose policies, regulations, or government funding decisions. For this study, we restricted PAOs to those that are based in the United States and submit Tax Form 990 because they have gross income greater than $200,000 or assets valued at more than $500,000.

PAO-funded medical product development activities include basic science research, preclinical research, and clinical trials funded by PAOs to support the development of biopharmaceutical drugs or medical devices, as well as development and maintenance of research tools such as patient registries and biobanks. PAOs can provide funding through partnerships with researchers at academic and medical institutions and life sciences companies, or they can conduct research in house. These research activities do not include (1) nonmonetary support for medical product development, such as facilitating patient involvement in research design or recruiting patients for clinical trials, or (2) research unrelated to medical product development, such as survivorship studies and clinical research on patient outcomes or behaviors.

Life sciences companies are pharmaceutical, biotechnology, and other for-profit companies that contribute to medical product development.

PAOs partner with academic and medical institutions and life sciences companies to advance medical product development activities. Studies exploring partnerships between these groups suggest that academic and medical institutions offer expertise in basic and translational research, experience in

clinical trial design, and access to clinical trial networks, whereas life sciences companies have demonstrated value in preclinical research, such as high throughput screening studies to test compounds for a biologic target and toxicology studies, and knowledge of regulatory standards and requirements (Ramsey et al. 2017; Kallio et al. 2023). PAOs often provide funding to these organizations to draw on their expertise. From 2008 to 2022, U.S. PAOs established nearly 700 funding arrangements with life sciences companies globally, totaling $2.4 billion; nearly three-quarters of these arrangements involved PAOs granting funding to life sciences companies, with most remaining arrangements establishing collaborative research partnerships between PAOs and life sciences companies. (IQVIA 2023).

PAOs typically fund medical product development activities through traditional grants but are increasingly using venture philanthropy arrangements. Traditionally, PAOs have used grants to provide research partners with a fixed amount of funding for specified research. Over the past couple of decades, PAOs have also started to use a venture philanthropy model, in which they invest in research and can receive financial returns from the products developed. PAOs use venture philanthropy to fund research that might be deemed too theoretical or risky for traditional grant-based funding (Lo and Thakor 2022; Shic et al. 2015). Although venture philanthropy agreements make up a small share of all relationships between U.S.-based PAOs and life sciences companies globally, the shift toward these types of arrangements has enabled many PAOs to sustain their philanthropy efforts and reinvest for their communities (IQVIA 2023). For example, the Cystic Fibrosis Foundation invested $150 million in drug development from 1998 to 2005, including a $40 million partnership with Vertex Pharmaceuticals that ultimately resulted in the discovery and approval of Kalydeco (ivacaftor), a drug that treats cystic fibrosis, in 2012. In 2014, The Cystic Fibrosis Foundation sold its royalty stream from its arrangement with Vertex Pharmaceuticals for $3.3 billion, which it is reinvesting in finding a cure for cystic fibrosis by partnering with a venture capital firm focused on gene therapies and gene editing (Giusti and Hamermesh n.d.). Other PAOs, such as Breakthrough T1D (formerly JDRF) and Alzheimer's Drug Discovery Foundation have seen returns of $46 million and $21 million, respectively, on their venture philanthropy investments in medical product development (Giusti and Hamermesh n.d.). An IQVIA study estimated that 2 percent of roughly 700 funding arrangements between PAOs and life sciences companies rely on this type of venture philanthropy approach where PAOs gain intellectual property as they invest (IQVIA 2023).

PAO funding can help overcome challenges in the research and development process. PAO funding can lower the barriers to entry in researching specific diseases or topics and entice other funders to support medical product development activities by advancing understanding of the underlying biology to derisk investments in these areas (Flotte 2015; Kim and Lo 2019; Lo and Thakor 2022). This is especially important for research on rare diseases, for which researchers often do not have sufficient funding and life sciences companies have a harder time making a business case for investments (Dunkle 2014; Litterman et al. 2014; Patterson et al. 2023).

PAOs have had noteworthy success in funding medical product development. Many PAOs have made a major impact on research on their condition of focus. For example, the Melanoma Research Alliance has invested over $150 million into translational research, which led to another $500 million in follow-on funding (Melanoma Research Alliance n.d.; Black and Brockway-Lunardi 2013). Investigators funded by the Melanoma Research Alliance have played a role in the development of 17 new FDA-approved treatments (Melanoma Research Alliance n.d.).

To better understand the medical product development landscape, more information is needed about PAOs' efforts to fund medical product development activities. For example, little is known about the number of PAOs that fund medical product development activities across nonprofit and for-profit partners. In addition, more research is needed on potential issues related to PAO funding of medical product development activities. PAOs that have a larger or wealthier donor base can fund more research than less-resourced PAOs, which could have an uneven effect on research productivity and clinical care (Farooq et al. 2020; National Academies of Sciences, Engineering, and Medicine 2023; Nori et al. 2023). The literature also highlights ethical considerations, including lack of transparency around PAOs' funding decisions, potential conflicts of interest when stakeholders belong to multiple groups (for example, when a current or former drug industry executive is on the board of a PAO), and whether PAOs' responsibilities change when they have a financial interest in commercializing a therapy through venture philanthropy (Megli 2024; Nguyen et al. 2022; Rahimzadeh et al. 2022; Winkler and Finegold 2007).

The Office of the Assistant Secretary for Planning and Evaluation contracted with Mathematica to study PAOs that fund medical product development activities (which we refer to as PAO funders). In the study, Mathematica sought to (1) identify the universe of PAO funders and (2) describe PAO funders' engagement in medical product development activities, including the types of activities they fund, the amount of funding they dedicate to these activities, and their approaches to funding such activities. Exhibit 2 lists the research questions that guided the study.

This report describes the methods used to identify PAO funders and develop a PAO funding engagement dataset (Section II); study findings, including the characteristics of PAO funders, types of medical product development activities funded by PAOs, and the arrangements used by PAO (Section III); and a discussion of results (Section IV). We also describe the methods used for a review of the literature on this topic (Appendix A) and provide additional documentation defining the variables in the PAO funding engagement dataset (Appendix B).

Exhibit 2. Research questions

This study addresses the following research questions:

1. How many PAOs fund medical product development activities for their disease?
2. What are the characteristics of PAOs that fund medical product development activities?
3. What types of medical product development activities (such as basic science, preclinical research, and clinical trials) are PAOs funding?
4. What is the range in magnitude of funding provided by PAOs for medical product development?
5. What approaches do PAOs use to fund medical product development activities?

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II. Methods

The study used a two-step process to identify and collect data on PAO funders. First, we compiled and reviewed a list of U.S.-based PAOs to determine which ones reported funding medical product development activities. Next, we used this list and publicly available data to develop a PAO Engagement dataset providing information about PAOs that reported funding medical product development. This section describes the methods used for both steps.

A. Methods used to identify PAO funders

To identify PAO funders, we developed a list of 1,555 PAOs that met the study criteria [that is, 501(c)(3) nonprofit organizations operating in the United States and filing Tax Form 990]. We then conducted searches for each PAO to determine whether it has funded medical product development activities.

1. PAO list development

First, we developed a list of 1,555 PAOs that operate in the United States, starting with the 1,215 PAOs in the Kaiser Health News (KHN) Pre$cription for Power dataset. This dataset includes a complete list of PAOs that (1) were operating and filed Tax Form 990 with the U.S. Internal Revenue Service in 2015 and (2) reported at least $500,000 in revenue. KHN developed the list using codes from the National Taxonomy of Exempt Entities, along with researcher reviews of organizations' mission statements.[2] To our knowledge, it is the only publicly available and ready-to-use list of the universe of PAOs.

To identify additional PAOs that are likely to fund medical product development activities and are newer or have lower revenue than those in the KHN Pre$cription for Power dataset, we conducted supplemental searches of the sources in Exhibit 3. For each source, we (1) compared the list of PAOs returned by the search to the Pre$cription for Power dataset to identify any PAOs missing from the latter; (2) confirmed that PAOs not in the Pre$cription for Power dataset met the criteria in our study definitions; and (3) if so, added these PAOs to our list.[3] These supplemental searches yielded an additional 340 PAOs, for a total of 1,555 PAOs.

[2] The method for developing the KHN Pre$cription for Power dataset is described here: Pre$cription For Power: The Patient Advocacy Database—KFF Health News.

[3] Besides confirming that each PAO met the criteria in the study definition (Exhibit 1), we also confirmed that the PAOs filed Tax Form 990 within the past two years (for calendar years 2022 or more recently) and reported positive revenue (>$0) on their most recent tax forms. We excluded PAOs that did not meet these criteria from our list for subsequent searching and categorization.

Exhibit 3. Supplemental data sources used to develop the list of PAOs

Besides the Kaiser Health News Pre$cription for Power dataset, we drew from the following data sources to identify an additional 340 PAOs in the United States:

- **Relevant peer-reviewed and gray literature** identified using the search protocol described in Appendix A. We uncovered the names of 35 PAO funders through these searches.
- **The National Organization for Rare Disorders (NORD) membership list**. NORD supports nonprofit organizations focused on rare diseases to help them serve their patients and families, educate medical professionals, and bring awareness to the general public. Their membership list includes more than 330 PAOs globally.
- **The Milken Institute's FasterCures TRAIN network membership list**. The TRAIN network is a group of more than 100 PAOs interested in taking a more strategic and entrepreneurial approach to their involvement in drug development research.
- **Tufts University's Patient Advocacy Leaders and Drug Development Industry Network** consortium. The consortium consists of 20 organizations, including PAOs and for-profit entities, engaged in improving collaboration between PAOs and biopharmaceutical companies.
- **IQVIA Institute's October 2023 report, *Supporting Patients Through Research Collaboration***. The report names more than 100 PAOs in exhibits and examples in the text.
- **The Rare Disease Clinical Research Network's** list of nearly 200 PAO partners

2. Search protocol to identify PAO funders

After we developed the list of 1,555 PAOs, we conducted a search for each PAO on the list to determine whether it funds medical product development activities. We reviewed PAOs' websites, including annual reports and impact reports, and most recent Tax Form 990 documentation using ProPublica's Nonprofit Explorer tool. Some PAO funders reported funding activities prominently on their home page—for example, the Michael J. Fox Foundation's home page highlights "over $1 billion funded in research programs for a cure to date." In other cases, this information was not as clear on the PAO's site, so we reviewed the PAO's most recent Tax Form 990, focusing on Part III.4 (description of accomplishments, including expenses and grants, for the organization's largest program services) and Schedule I (list of grants and other assistance to domestic organizations and governments of over $5,000, including recipient, amount, and purpose) to look for evidence that the PAO funded medical product development activities. Exhibit 4 shows an example of Part III of Tax Form 990 for a PAO that highlighted medical product development activities.

Exhibit 4. Example of Tax Form 990 for a PAO highlighting medical product development activities

Part III Statement of Program Service Accomplishments

Check if Schedule O contains a response or note to any line in this Part III . . . . . ☐

1 Briefly describe the organization's mission:

KIF1A.ORG IS A GLOBAL COMMUNITY DEDICATED TO IMPROVING THE LIVES OF THOSE AFFECTED BY KIF1A-ASSOCIATED NEUROLOGICAL DISORDERS AND ACCELERATING RESEARCH TO FIND A CURE.

2 Did the organization undertake any significant program services during the year which were not listed on the prior Form 990 or 990-EZ? . . . . . ☐ Yes ☑ No

If "Yes," describe these new services on Schedule O.

3 Did the organization cease conducting, or make significant changes in how it services? . . . . . ☐ Yes ☑ No

If "Yes," describe these changes on Schedule O.

4 Describe the organization's program service accomplishments for each of its Section 501(c)(3) and 501(c)(4) organizations are required to report the am easured by expenses. rs, the total expenses, and revenue, if any, for each program service reported.

The description highlights the PAO's commitment to "translational research to rapidly discover treatment...and accelerate therapeutic development".

4a (Code:) (Expenses $ 159,067 including grants of $ 126,432) (Revenue $ 0)

RESEARCH AND DEVELOPMENT - KIF1A.ORG POWERS COLLABORATIVE AND TRANSLATIONAL RESEARCH TO RAPIDLY DISCOVER TREATMENT FOR KAND. KIF1A.ORG MANAGES SEVERAL PROGRAMS TO DE-RISK AND ACCELERATE THERAPEUTIC DEVELOPMENT, INCLUDING THE KIF1A RESEARCH NETWORK, DIRECT RESEARCH FUNDING, AND OPEN-ACCESS TOOLS FOR DEVELOPMENT, SUCH AS NATURAL HISTORY DATA, DISEASE MODELS, AND BIOMARKERS.

4b (Code:) (Expenses $ 125,168 including grants of $ 1,676) (Revenue $ 0)

KAND FAMILY AND SCIENTIFIC ENGAGEMENT CONFERENCE - KIF1A.ORG HOSTED THE 2023 KAND FAMILY & SCIENTIFIC ENGAGEMENT CONFERENCE IN NEW YORK CITY, NY FROM AUGUST 3-6, 2023. THESE CONFERENCES BRING TOGETHER KIF1A.ORG COMMUNITY MEMBERS IN ONE SETTING INCLUDING PATIENTS, FAMILIES, SCIENTISTS, CLINICIANS, BIOTECH LEADERS, AND SUPPORTERS. THE LATEST 2023 UPDATES IN RESEARCH AND THERAPEUTIC DEVELOPMENT WERE SHARED AND PATIENTS AND FAMILIES SHARED THEIR PERSONAL STORIES WITH KAND. THIS 4 DAY CONFERENCE ALSO INCLUDED RESEARCH PARTICIPATION FOR 30 KAND PATIENTS AND FAMILIES AND DOUBLED OUR INPERSON KOALA ASSESSMENTS.

4c (Code:) (Expenses $ 23,525 including grants of $ 1,848) (Revenue $ 0)

OTHER PROGRAMS - GENERAL, COMMUNITY AND FAMILY SUPPORT, CONFERENCE ATTENDANCE AND KOALA TRAVEL REIMBURSEMENT

Note: The example shows the 2023 Tax Form 990 from KIF1A.ORG.

Some PAOs required additional consideration to determine whether they funded medical product development. For these nuanced cases, we decided to exclude the following PAOs from the list of medical product development funders:

- **Local chapters of PAOs that do not fund medical product development outside of contributions to their national organization.** This arrangement between local chapters and their parent PAOs was common, as exemplified by local chapters of the Epilepsy Foundation of America, Alzheimer's Disease and Related Disorders Association, and Lupus Foundation of America, among others. In contrast, in a few cases, chapters reported funding medical product development with local partners—for example, the Illinois chapter of the National Kidney Foundation provided research grants to the University of Illinois and Northwestern University for basic science and translational research. These cases are included in the subset of PAOs that fund medical product development.
- **PAOs that funnel research funding through other PAOs, rather than funding research partners directly.** For example, the Bear Necessities Pediatric Cancer Foundation seeks "to eliminate pediatric cancer by supporting cutting-edge research." The PAO does this by providing funding to Alex's Lemonade Stand Foundation, a PAO known for "rigorous selection of research grants." Because Alex's Lemonade Stand Foundation is included in our list, we capture the funding only once.
- **PAOs that exclusively provide nonmonetary support for medical product development (such as research design consultations or clinical trial recruitment) instead of monetary support.** Many PAOs partner with research institutes and life sciences organizations to provide nonmonetary support

for clinical trial recruitment or study design consultation; we excluded these PAOs unless they also provided evidence of funding medical product development.

- **PAOs that fund research unrelated to medical product development.** For example, the Cancer Support Community funds behavioral and survivorship research "to shed light on the experiences of patients living with and beyond cancer."

Of the 1,555 PAOs on the list, 585 (38 percent) reported funding medical product development activities. This included 386 of the 1,215 PAOs on the Pre$cription for Power list (32 percent) and 199 of the 340 PAOs that we added to the Pre$cription for Power list through nonsystematic searches (59 percent).

B. Methods used to develop a PAO funding engagement dataset

After identifying the subset of 585 PAOs that reported funding medical product development, we examined each PAO funder to learn more about its engagement in medical product development activities. We spent up to 15 minutes per PAO reviewing PAOs' tax documentation and websites to populate a dataset with variables related to the following topics:

- Types of medical product development activities that PAOs fund
- Amount of funding PAOs provide for medical product development activities
- Types of funding approaches PAOs use to engage in medical product development activities

We also recorded select characteristics from PAOs' most recent Tax Form 990 filing, including total annual revenue from their most recent tax form (usually 2022 or 2023) and the year that they were first recognized as a tax-exempt organization, which we use as a proxy for the year that the PAO was first established.

Below, we describe our approach to populating the dataset and note data limitations for each group of variables. Appendix B provides additional details on the variables in the PAO engagement dataset and their specifications.

1. Types of medical product development activities PAOs fund

For each type of medical product development activity—basic science research, preclinical research, clinical trials, and registry or biobank development and maintenance—we reviewed available information to determine whether the PAO reported funding the activity (either by conducting these research activities internally or through granting funds to external partners), or whether it was not reported or unclear (Exhibit 5). Organizations that provide grants to external organizations often listed current and previously funded studies on their websites, enabling the research team to see whether PAOs directed funds toward specific activities. For instance, the list of funded studies on Race to Erase MS's website includes basic science and preclinical trials but does not mention clinical trials. Other organizations list the purpose of grants in Section I of Tax Form 990, although the level of detail PAOs included varied.

2. Amount of funding PAOs provide

We collected and populated data for three variables related to the amount of funding PAOs provide for medical product development: (1) an open-ended field for entering details about funding amounts for medical product development activities; (2) an interim variable for the annual funding for medical product development activities for the most recent year available or, if this was not available, the estimated amount;[4] and (3) a categorical variable to identify PAOs that provide low levels of funding for medical product development activities (less than $100,000 per year), medium levels ($100,000 to $999,999), and high levels (more than $1,000,000).

Exhibit 5. Study definitions of medical product development activities

We defined medical product development activities as follows:

- **Basic science research:** research to improve understanding of health conditions, such as natural history studies and biomarker discovery.
- **Preclinical research:** translational medicine studies, such as *in vivo* studies that assess medical product candidates on animal models and *in vitro* studies that assess medical product candidates on cell-based models.
- **Clinical trials:** all phases of trials with human participants to test new medical products.

In addition, we documented whether a PAO reported **funding or developing a patient registry or biobank**. Appendix B contains additional information about our approach to assessing medical product development activities. ◢

3. Types of funding approaches PAOs use

We documented PAOs' approaches to funding medical product development, including funding external partners and conducting in-house research and development activities (Exhibit 6).

For each approach, we recorded whether the PAO reported using the approach, or whether it was unclear or not reported. Many PAO websites featured lists of grant awardees and funded research partners at academic and medical institutions or highlighted the research milestones of their internal staff if they conducted in-house product development activities. When websites had limited information, tax documentation usually included a list of organizations to which the PAO

Exhibit 6. Study definitions of funding approaches PAOs use

We defined funding approaches as follows:

Providing funding to external partners, including:

- Funding academic and medical institutions
- Funding life sciences companies

Conducting in-house research and development activities, including:

- Conducting early-stage research (such as natural history studies) using in-house staff
- Conducting clinical trials at service delivery sites operated by the PAO
- Operating as a nonprofit life sciences company with PAO-owned labs and staff
- Other approaches involving substantial internal financial investments to advance medical product development ◢

[4] For PAOs that did not report funding for the year but noted medical product development funding to date (for example, the Amyotrophic Lateral Sclerosis Association reported funding over $154 million in drug development activities since 2014), we calculated the average funding amount per year, not accounting for inflation or economic factors, though the true amounts likely varied year to year.

granted funding for amounts over $5,000 and listed the purpose of the grant. Exhibit 7 shows an example of how some PAOs report grants to research partners on Tax Form 990 Schedule I.

Exhibit 7. Example of Tax Form 990 for a PAO that provides grants to life sciences companies

Part II **Grants and Other Assistance to Domestic Organizations and Domestic Governments.** Complete if the organization answered "Yes" on Form 990, Part IV, line 21, for any recipient that received more than $5,000. Part II can be duplicated if additional space is needed.

(a) Name and address of organization or government	(b) EIN	(c) IRC section (if applicable)	(d) Amount of cash grant	(e) Amount of non-cash assistance	(f) Method of valuation (book, FMV, appraisal, other)	(g) Description of noncash assistance	(h) Purpose of grant or assistance
(1) Columbia University PO Box 29789 General Post office New York, NY 100879789			50,000	0	contractual amount		This study will test the diagnostic potential of extracellular vesicles (EVs) released by microglia. Microglia are resident immune cells in the central nervous system (CNS) which perform multiple functions including, immune response to local environment, communication with all CNS cells (neurons, astrocytes and oligodendrocytes) and phagocytic clearance of misfolded proteins and apoptotic or necrotic cells. EVs derived from microglia can play an important role as mediators of both pathological and reparative mechanisms in MS. Microglia are known to play key roles in both active inflammation and remyelination in MS
(2) Nanosomix Inc 4 Little Pond Laguna Niguel, CA 92677			10,000		contractual amount		Nanosomix analyzed samples for MRF

Grant to academic institution

Grant to life sciences company

Note: The example shows the 2022 Tax Form 990 from Myelin Repair Foundation Inc.

C. Limitations

Although we attempted to develop a comprehensive list of PAO funders and a robust dataset describing PAOs' engagement in funding medical product development activities, our methodology had several limitations.

We did not conduct a systematic search of all PAOs in the U.S. The study leveraged existing PAO lists to streamline the search process and increase efficiency, but it did not include a systematic search of all PAOs in the United States.[5] Other studies reported over 3,000 PAOs in the United States, which suggests our list of PAOs undercounts the universe of PAOs (IQVIA 2023). However, we believe that the PAO list we compiled captures most of the major players in medical product development, as we would expect these to be PAOs with moderate to high annual revenue or PAOs involved in the initiatives and groups in Exhibit 3, which we used to inform our nonsystematic supplemental search for PAOs.

The PAO Engagement Dataset may underestimate the total number of PAO funders in the United States. We expect that the dataset excludes some PAO funders – especially those that are less involved in funding medical product development activities – given that (1) the overarching list of U.S. PAOs used to guide the search process was not exhaustive, and (2) the approach to identify PAO funders was limited to

[5] A systematic search of all PAOs in the U.S. would have been methodologically preferable to the non-systematic approach used. While there was not a feasible systematic search strategy for this study, we made a good faith effort to find PAOs engaged in funding medical product development activities.

publicly available information on PAOs' websites and in their tax documentation. Although some PAOs clearly described their engagement in funding medical product development activities on their websites or tax documentation, neither source perfectly captures PAO funding of these activities across the cohort of PAO funders. In other cases, PAO funders may have reported this information in a way that evaded our search protocol due to the lack of standardized reporting of this information.

Limitations in publicly available information affected our ability to identify funding activities and funding arrangements among known PAO funders. It is likely that we undercounted the number of PAOs funding each type of activity or engaging in the various funding arrangements because they did not report it on their website or Tax Form 990, or we were unable to find the information during our search. For example, for 70 of 585 PAO funders (12 percent), we were unable to find any information on funded activities beyond a blanket statement that the PAO funded medical product research. Another data element that was affected by limitations in publicly available information is the number of PAOs funding life sciences companies. Many life sciences companies are not based in the United States and would therefore not be listed in the tax filings among PAOs' domestic grant recipients (and PAOs are not required to list grants to international organizations at the organization level in their tax forms; this information is required only at the country level). As a result, we often had to draw this information from PAOs' websites, which were not always transparent about funding arrangements with life sciences partners.

There were several challenges in estimating the amount of funding that PAOs dedicate to medical product development activities. These include:

- *Variation in reporting periods*. Some PAOs reported annual medical product development funding, whereas others described the amount of funding invested since the PAO's inception. When PAOs provided funding totals since inception, we calculated an average annual amount, but we did not account for fluctuation by year, inflation, or other economic factors.
- *Inclusion of unrelated research in estimates*. Some PAOs funded research unrelated to medical product development in addition to qualifying research and, in many cases, reported only their overall research funding amounts. For example, the Susan G. Komen Breast Cancer Foundation funds several studies on how changes in diet and lifestyle might prevent cancer, along with projects focused on developing new cancer treatments. We tried to exclude these unrelated studies from our calculations, but it was not always apparent or feasible. Thus, funding estimates may inadvertently include research that is unrelated to medical product development.
- *Missing data*. In the dataset, funding amount is missing for 11 percent of PAO funders (65 of 585 PAOs). Some PAOs, such as the Prevent Cancer Foundation, listed a total funding amount on their websites, yet it was clear that a significant portion of their funded projects fall outside of our scope; in these cases, we did not include a total funding amount in the data set. In other cases, this information was not available. This was most common among PAOs that primarily conduct research in house and did not clearly articulate spending on medical product development, newer PAOs that did not have detailed websites, and PAOs that focus on unrelated activities (such as education for patients and medical professionals) but provide some funding for medical product development activities.

Due to these limitations, we recommend dataset users focus on the categorical annual funding variable instead of the interim continuous variable, and look at broad trends, such as the distribution and range of funding provided by PAOs, rather than details, such as the amount of funding provided by a PAO or total amount of funding dedicated by PAOs for medical product development. In the categorical variable, PAOs on the cusp of categories are more likely to be miscategorized than other PAOs.

Given these limitations, the counts presented throughout this report – including the number of PAO funders, number of PAO funders engaged in specific activities, and number of PAOs using different funding arrangements – should be interpreted as a minimum rather than the true number of PAOs engaging in this work. This information can provide insight into the characteristics of PAO funders, such as their average revenue, and high-level trends in PAO funders' engagement in medical product development, such as which activities PAO funders are most and least likely to fund.

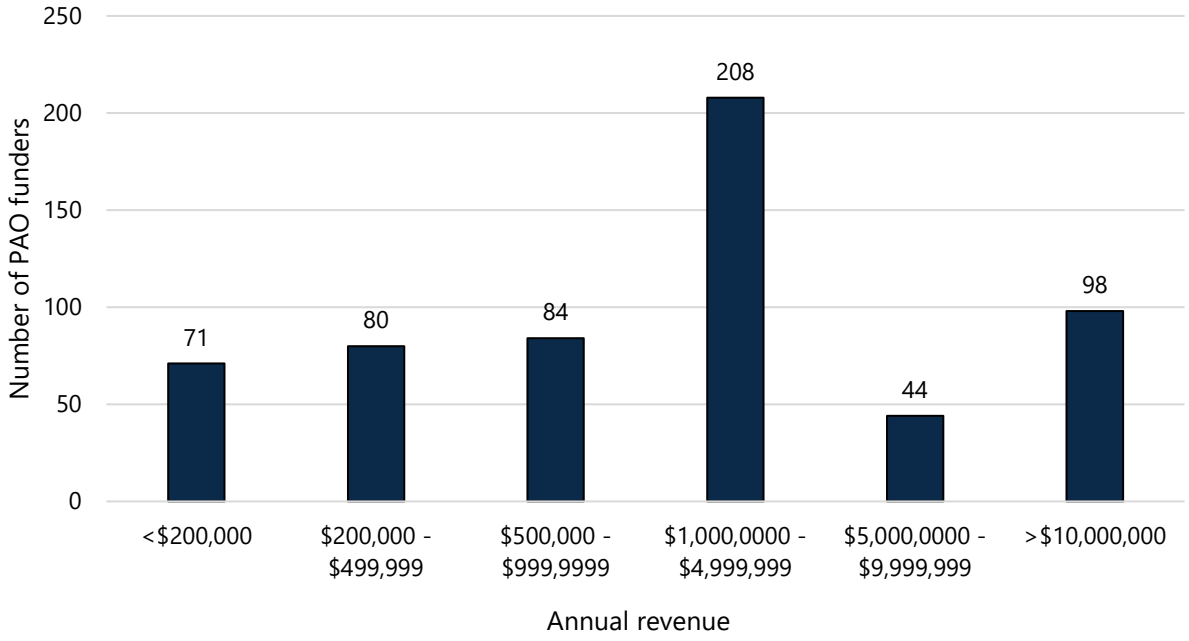
III. Findings

Of the 1,555 PAOs identified, 585 (38 percent) reported funding medical product development activities. This section summarizes our findings about PAO funders.

A. Characteristics of PAOs that fund medical product development

The majority of PAOs that fund medical product development activities have an annual revenue greater than $1 million (60 percent; 350 of 585 PAOs), although a quarter of PAO funders (151 of 585 PAOs) reported less than $500,000 in annual revenue. Among the PAOs that we identified as funding medical product development activities, the median annual revenue was $1,527,339. Nearly one-fifth of PAO funders (17 percent; 98 of 585 PAOs) reported more than $10 million in annual revenue, with a maximum reported revenue of $925,828,648 (the American Heart Association). In the aggregate, this amounted to more than $8.5 billion in annual revenue among PAOs that reported funding medical product development. While many PAOs were relatively large, approximately one-quarter of PAO funders (26 percent; 151 of 585 PAOs) reported an annual revenue of less than $500,000, and 12 percent (71 out of 585 PAOs) of PAO funders reported an annual revenue of less than $200,000 (Exhibit 8).

Exhibit 8. PAO funders' annual revenue, by number of PAOs (N = 585)



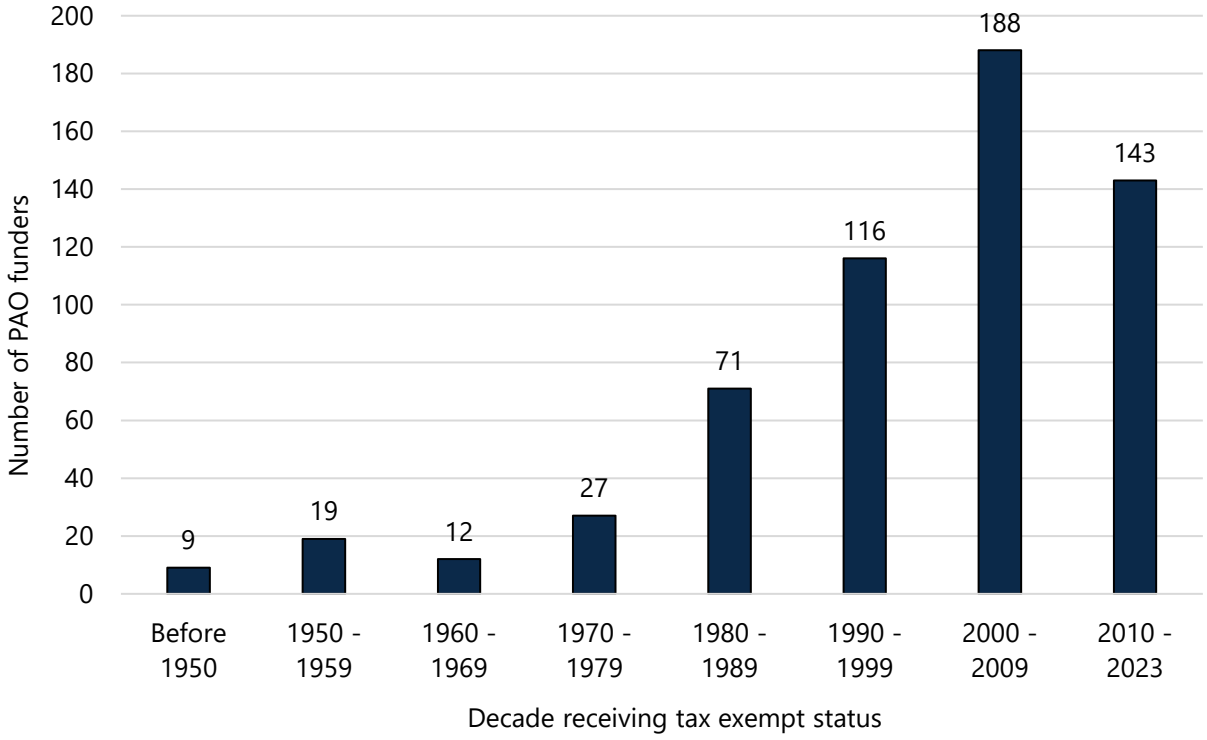
PAOs with the highest revenue are generally older and more established than those with lower revenue. The PAO funders identified in this study reported first being recognized as a tax-exempt organization from 1937 to 2023, with the median year of receiving tax exempt status being 2001. Among PAOs reporting $1 million to $9.9 million in annual revenue, the median year of tax-exempt status was 2001, while among those reporting more than $10 million in revenue, it was 1993 (Exhibit 9).

Exhibit 9. Median and range of year of receiving tax exempt status, by PAO funders' annual revenue

Included PAOs	Median year of receiving tax exempt status	Range of year of receiving tax exempt status
All PAOs (N = 585)	2001	1937 – 2023
PAOs with < $1 million revenue (n = 235)	2008	1955 – 2023
PAOs with $1 million – $9.9 million revenue (n = 252)	2001	1937 – 2018
PAOs with >$10 million revenue (n = 98)	1993	1942 – 2020

While the oldest PAO funders that we identified date back to 1937, approximately one-quarter of currently operating PAO funders have emerged in the past 15 years. Of the 585 PAOs that reported funding medical product development activities, 24 percent (143 PAOs) reported being recognized as tax-exempt organizations in 2010 or later (Exhibit 10). An IQVIA study suggests that the focus and role of PAOs have evolved over the years: many older PAOs originally focused on helping patients who were deaf or blind or had major disorders like cancer or infectious diseases, whereas starting in the 2000s, more PAOs emerged with missions to find cures for specific diseases (IQVIA 2023).

Exhibit 10. PAO funders' year of receiving tax exempt status through 2023, by number of PAOs (N = 585)

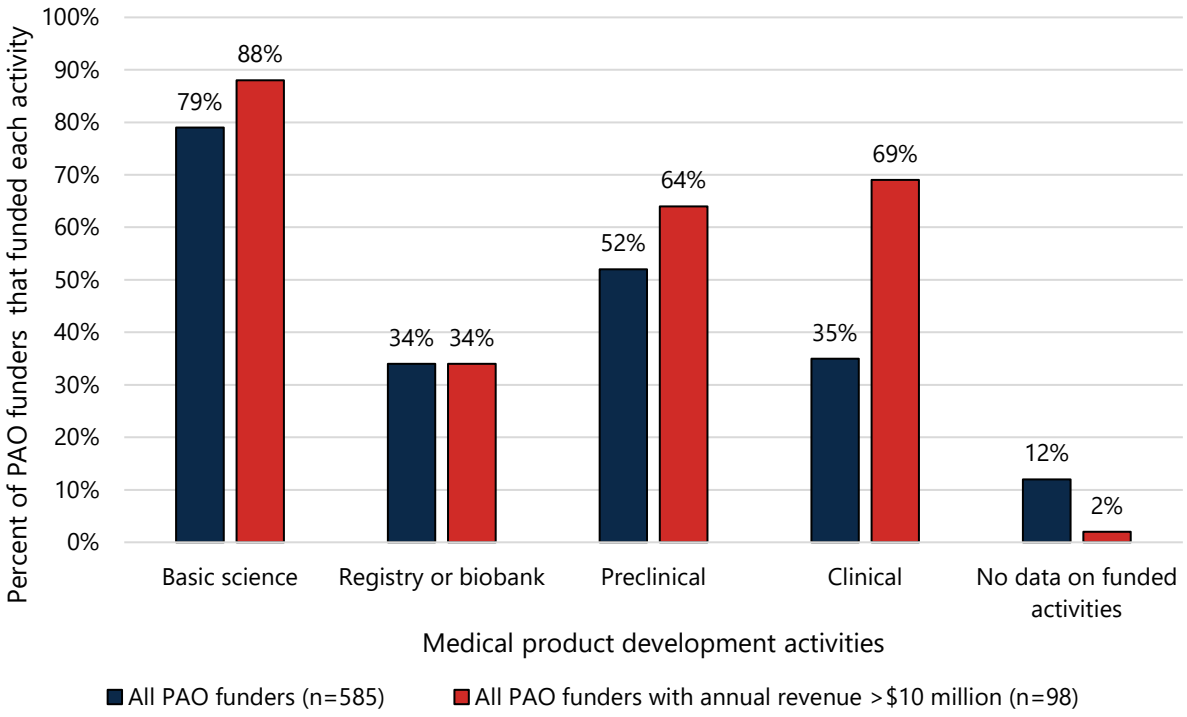


B. Medical product development activities funded by PAOs

Exhibit 11 summarizes the distribution of PAOs that fund various types of medical product development activities, among all PAOs and PAOs with more than $10 million in annual revenue. PAO funders were most likely to report funding basic science research, followed by preclinical research,

and finally clinical trials. These findings align with past studies of PAOs' focus areas for medical product development activities. A study on financial arrangements between PAOs and life sciences companies showed that basic science or discovery research was the most common area of investment (representing 41 percent of deals between PAOs and life sciences companies), followed by preclinical research (30 percent of deals); Phase I or II clinical trials (19 percent of deals); and later-stage clinical trials, other clinical research, and other unspecified activities (10 percent of deals) (IQVIA 2023).

Exhibit 11. Percentage of PAO funders that report funding medical product development activities, by type of activities funded and annual revenue



Basic science research, such as natural history studies and research to identify biomarkers, was the most common activity that PAOs funded. Seventy-nine percent of PAO funders (462 of 585 PAOs) funded basic science research. PAOs often reported providing grants to external researchers for this type of research. For example, the Cancer Prevention Initiative, Inc., stated on its 2021 Tax Form 990 that it provided $150,000 to the Cleveland Clinic to "identify proteins uniquely and highly expressed in breast cancer of BRCA1 mutation carriers," with the goal of using newly identified proteins as the basis for developing preventative vaccines. In some cases, PAOs reported conducting basic science research in house—for example, the FOXG1 Research Foundation has a small in-house research team leading a natural history study, in addition to providing grants to external research teams.

To support basic science research, 34 percent of PAO funders (198 of 585 PAOs) reported on their website or in their tax documentation that they funded patient registries or biobanks.[6] Most of these PAOs developed and maintained registries and biobanks, although several reported funding external partners to develop and maintain these resources. For example, the Fibromuscular Dysplasia Society of America, Inc., reported on its 2022 Tax Form 990 that it granted $50,000 to the University of Michigan to serve as the coordinating center for the Fibromuscular Dysplasia Patient Registry. Many of the PAOs that funded patient registries and biobanks focus on rare diseases (such as the Oxalosis and Hyperoxaluria Foundation, Pachyonychia Congenita Fund, and the Mowat-Wilson Syndrome Foundation). This finding aligns with a 2023 IQVIA Institute study showing that 159 PAOs in the United States maintained patient registries in 2021, and of these, 63 percent were PAOs focused on rare diseases (IQVIA 2023).

The second most common type of activity was preclinical research, such as *in vivo* or *in vitro* studies. Fifty-two percent of PAO funders (307 of 585 PAOs) reported funding preclinical research. For example, the STXBP1 Foundation reported awarding $25,000 in 2024 to a lab at the University of Richmond to conduct a drug repurposing study on zebrafish to identify potential therapeutics for STXBP1-associated epilepsies.

Fewer PAOs funded clinical trials (35 percent; 205 of 585 PAOs). In general, PAOs that funded clinical trials either reported funding early-stage clinical trials (Phase I or Phase II) or did not specify which phases they fund, and most PAOs funding clinical trials also funded basic science or preclinical research (94 percent; 192 of 204 PAOs). For example, the Pediatric Brain Tumor Foundation has funded several trials, including a Phase I trial on the impact of combining two drugs, trametinib and everolimus, to treat children with recurrent gliomas. This count also includes several PAOs that used in-house staff and resources to conduct clinical trials at their service sites. For example, the House Institute Foundation, which focuses on hearing and neurological disorders, is currently conducting a trial to determine whether the allergy medication montelukast can treat symptoms of Meniere's disease.

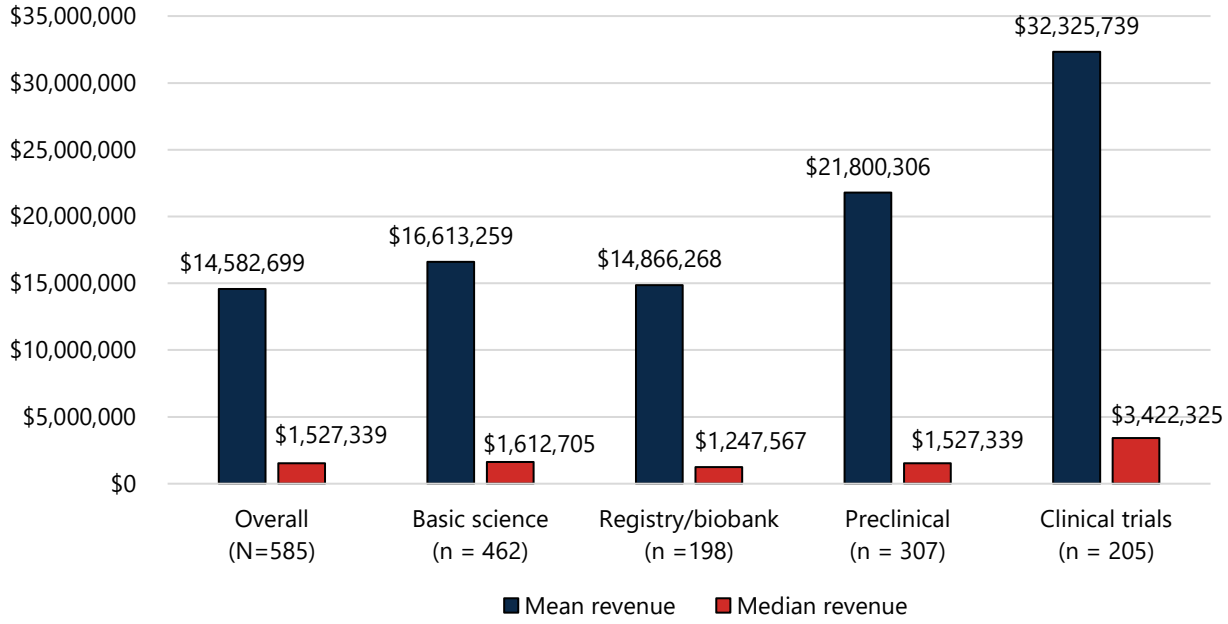
PAO funders with higher revenue were more likely than those with lower revenue to report funding medical product development activities—especially clinical trials. Among the PAO funders with more than $10 million in annual revenue, 88 percent (86 of 98 PAOs) funded basic science research compared to 79 percent (462 of 585 PAOs) overall, and 64 percent (63 of 98 PAOs) funded preclinical research compared to 52 percent (307 of 585 PAOs) overall. Most notably, PAOs with more than $10 million annual revenue were more than twice as likely to fund clinical trials than PAOs overall: 69 percent of PAOs with more than $10 million annual revenue (68 of 98 PAOs) funded clinical trials compared to 35 percent (205 of 585 PAOs) overall.

Relatedly, the median annual revenue of PAOs that reported funding clinical trials was more than twice as high as PAO funders overall (Exhibit 12). The median annual revenue among PAOs that reported funding clinical trials was $3,422,325 compared to $1,527,339 among PAO funders overall. There are likely several reasons for this trend. First, clinical trials are expensive to administer. A 2014 report from

[6] Registries enable researchers to evaluate the outcomes of a population affected by a particular condition over time and understand the natural history of a disease. Biobanks support detection or discovery of biomarkers or genetic variants (IQVIA 2023).

the Office of the Assistant Secretary for Planning and Evaluation showed the average cost of clinical trials is $4 million for Phase I, $13 million for Phase II, and $20 million for Phase III (Sertkaya et al. 2014). A separate study by the Institute for Safe Medication Practices revealed that the median cost of clinical trials for drugs approved by the Food and Drug Administration between 2015 and 2016 was $19 million, with the least expensive costing $2.1 million (Moore et al. 2018). Although researchers might have several sources of funding, the cost of clinical trials likely causes lower-revenue PAOs to focus their funding on other activities. Second, many PAOs that conduct clinical trials also provide direct patient services, such as Mary M. Gooley Hemophilia Center, Inc., which might elevate their revenue and make it easier to afford the cost of funding clinical trials, as well as help recruit patients. Finally, some lower-revenue PAOs might focus on diseases that are less researched and thus further away from the clinical trial stage. This might often be the case for PAOs focused on rare diseases, such as the Foundation for USP7-Related Diseases and Hope in Focus, Inc., where researchers are often still working to understand the mechanisms of the diseases before they can begin developing drugs and pursuing clinical trials.

Exhibit 12. Mean and median revenue among PAOs that fund different types of medical activities



Note: Categories are not mutually exclusive. PAOs may be included in multiple categories if they reported funding multiple types of activities.

PAOs that reported funding registries or biobanks were, on average, more recently established as tax-exempt organizations than PAO funders overall. Although year of receiving tax exempt status was not associated with trends in funding basic science research, preclinical research, or clinical trials, the median year in which PAOs that reported funding registries or biobanks received a tax exempt status was 2005 compared to 2001 for PAO funders overall. Many of these PAOs focus on rare diseases or gene mutations that have been discovered within the past couple decades, such as DDX3X gene mutations, KAT6-related disorders, and megalencephaly-capillary malformation-polymicrogyria (MCAP), so the PAOs are likely filling an unmet need by funding and developing registries.

C. Amount of PAO funding dedicated to medical product development activities

PAOs varied widely in the amount of funding they provide for medical product development activities. PAOs reported dollar amounts that ranged from $2,000 per year to over $300,000,000 annually. Of the 520 PAOs with nonmissing data related to the amount of funding they dedicate to medical product development research, 24 percent (123 PAOs) funded less than $100,000 per year on average; 48 percent (247 PAOs) funded $100,000 to $999,999; and 29 percent (150 PAOs) funded more than $1 million. An additional 65 PAOs did not report any information about the amount of funding they provide for medical product development beyond reporting that they have funded these activities in the past. These PAOs included small PAOs that did not have websites detailing their funding contributions, as well as large PAOs that conduct a variety of activities (such as advocacy and service provision) and do not specify the breakdown of funding to the different programs they support on their websites or tax documentation.

In general, PAOs with higher revenues also dedicated more funding to medical product development activities. PAOs that reported spending less than $100,000 on medical product development activities annually have a median revenue of $351,804, whereas the median revenue for PAOs spending over $1,000,000 annually is $8,841,952 (Exhibit 13).

Exhibit 13. Level of medical product development funding provided by PAO funders, by average PAO revenue

PAO's annual funding for medical product development activities (N = 585)	Mean PAO revenue	Median PAO revenue
Less than $100,000 (n = 123)	$1,859,463	$351,804
$100,000–$999,999 (n = 247)	$3,871,061	$1,317,262
At least $1,000,000 (n = 150)	$45,287,501	$8,841,952
No data/missing (n = 65)	$8,505,966	$1,108,820

D. Funding arrangements used by PAOs

Exhibit 14 shows the distribution of PAO funders by the types of funding arrangements they reported using to advance medical product development activities.

Exhibit 14. Number and percentage of PAO funders using various types of funding arrangements, by type of arrangement

Type of funding arrangement	Number of PAO funders (N= 585)	Percentage of PAO funders
Funds external organizations		
Funds academic or medical institutions	536	92%
Funds life sciences companies	106	18%
Conducts in-house research and development activities		
Conducts basic science research	82	14%
Conducts clinical trials at service sites	9	2%
Operates as a nonprofit life sciences company	11	2%
Conducts other substantial in-house activities	14	2%

Note: PAOs might engage in multiple funding arrangements and are included in totals for each arrangement.

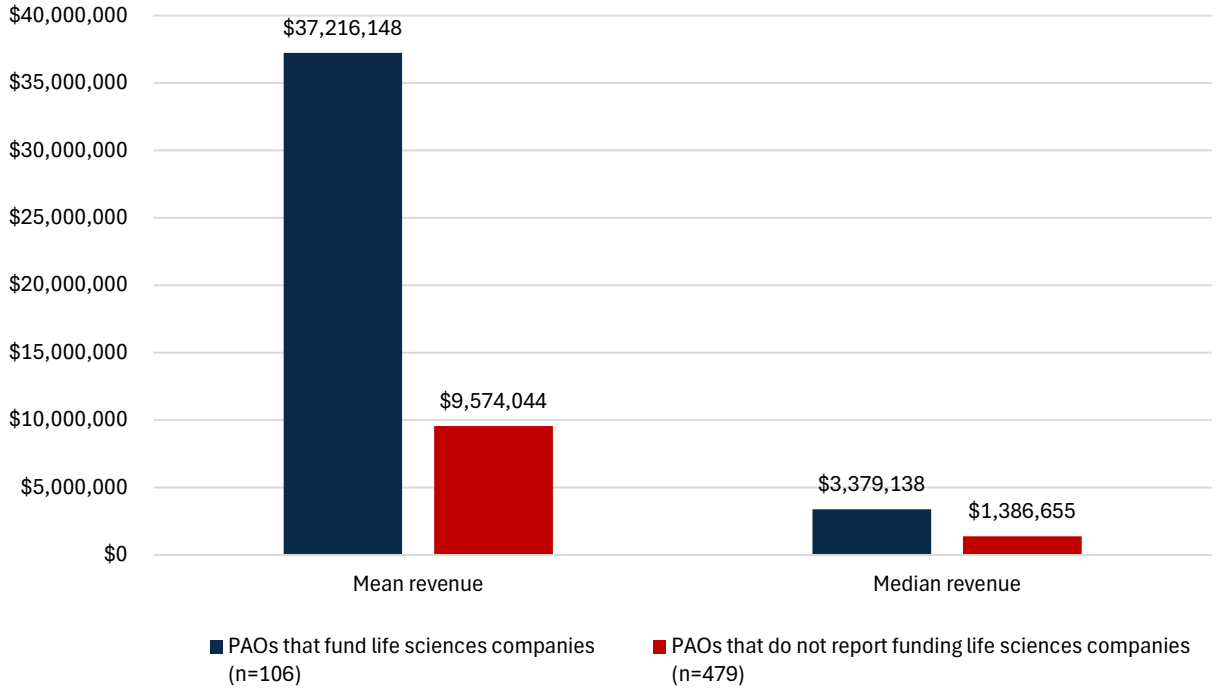
1. Funding external organizations

Ninety-two percent of PAO funders (536 of 585 PAOs) reported funding academic or medical institutions, typically through competitive grant processes. Most PAOs described convening scientific advisory committees to help select grantees that would further the PAO's research goals. For example, the Propionic Acidemia Foundation noted in the "Grants" section of its website that it awards $3,000 to $50,000 grants for research projects that it selects based on "scientific validity and merit; technical feasibility; and impact on accelerating discovery, development, or evaluation of therapeutics." PAOs often described these opportunities as "seed grants" intended to garner additional financial backing from life sciences companies and the federal government. For example, the Breast Cancer Alliance's website said the PAO prides itself on funding early-stage research to "get from the conceptual stage to eligibility for federal support."

Fewer PAO funders (18 percent; 106 of 585 PAOs) reported funding life sciences companies directly. For example, CureLGMD2i reported on its Tax Form 990 that it granted $100,000 each to two life sciences companies—Myogenica and Kinea Bio—to research a cure for limb-girdle muscular dystrophy in 2022. Among PAOs that fund life sciences companies, many provided grants, whereas fewer reported pursuing venture philanthropy arrangements in which the PAO has a stake in the intellectual property rights of medical product discoveries. Nearly all PAOs that fund life sciences companies (97 percent; 103 of 106 PAOs) said they also provide funding to academic and medical institutes to support medical product development. That said, our dataset may underestimate the number of PAOs funding life sciences companies (see Section II.B).

PAOs that reported funding life sciences companies had substantially higher revenue than those that do not fund life sciences companies or where this could not be determined (Exhibit 15). The 106 PAOs that reported funding life sciences companies have a median annual revenue of $3,379,138, which is more than twice the median annual revenue of the 479 PAOs that did not report funding life sciences companies or where this could not be determined ($1,386,655). Only 15 percent of PAOs that reported funding life sciences companies (16 of 106 PAOs) have an annual revenue of less than $500,000, compared with 28 percent (135 of 479 PAOs) that do not report funding life sciences companies. Though these PAOs are, on average, larger, several smaller PAOs reported funding life sciences companies directly. For example, the Malan Syndrome Foundation, which was first recognized as tax exempt in 2019 and had an annual revenue of less than $200,000 in 2023, reported granting $140,000 to Invivo Biosystems to use its preclinical screening technology to conduct a drug repurposing study to explore a cure for Malan syndrome. Similarly, Kif1A.Org—another PAO that has emerged within the past decade and had a relatively modest revenue of $230,000 in 2023—reported funding NeuCyte, an early-stage biotechnology company focused on neurological diseases, to develop cell models of KIF1A Associated Neurological Disorder and test potential therapeutics.

Exhibit 15. Mean and median revenue among PAO funders that do and do not report funding life sciences companies



2. Conducting in-house research and development activities

Another less common approach to funding medical product development activities was to conduct research and development in house using PAO staff. PAO funders described a variety of in-house arrangements, including hiring staff to conduct and support early-stage research, such as natural history studies; conducting clinical trials at service delivery sites operated by the PAO; operating primarily as nonprofit biotechnology companies with PAO-funded labs and staff; and providing substantial financial resources to support other in-house medical product development activities.

Fourteen percent of PAO funders (82 of 585 PAOs) reported using PAO staff and resources to conduct their own early-stage research, such as basic science research and development and maintenance of patient registries or biobanks. For most of these PAOs, these expenditures were limited (for example, having one or two researchers on staff to oversee a patient registry or conduct a natural history study using the patient registry), although several PAOs described larger-scale in-house research operations to supplement the research that they fund with external partners. For example, the Chordoma Foundation reported having its own in-house research lab with its own collection of cell lines and mouse models, which they use for biomarker discovery and other experiments. Three-quarters of PAOs that reported conducting in-house research (63 of 81) also said they provided funding to external partners for medical product development activities, although a few smaller PAOs, such as the Snyder-Robinson Foundation and the International WAGR Syndrome Association, reported maintaining registries but did not otherwise report funding or conducting medical product development activities.

Two percent of PAO funders (nine of 585 PAOs) reported conducting clinical trials at the PAOs' service delivery sites. For example, the Community Research Initiative of New England, Inc., website said the PAO has a "dedicated research team [that] runs clinical trials to discover safer, simpler, and more effective treatments for HIV and other infectious diseases." These PAOs also tend to be older than other PAOs. Among these nine PAOs, the median year of receiving their tax-exempt status was 1994, as opposed to 2001 for all PAO funders.

Two percent of PAO funders (11 of 585 PAOs) reported operating primarily as nonprofit life sciences organizations with their own research staff and labs.[7] One example is Cohen Veterans Bioscience, "a biomedical research and technology company dedicated to advancing brain health by advancing precision diagnostics and tailored therapeutics." Another example is the ALS Therapy Development Institute, which described itself as "the largest drug discovery lab in the world focused solely on finding treatments for ALS". The ALS Therapy Development Institute's' website highlights that, unlike for-profit life sciences companies that need to be responsive to investors, they "are only answerable to our ALS community stakeholders…and only worry about finding effective treatments for ALS rather than providing a financial benefit to shareholders."

Two percent of PAO funders (14 of 585 PAOs) described using substantial financial resources to support other in-house medical product development activities. For example, the International Myeloma Foundation reported hiring an in-house research team to lead a "multi-stakeholder research consortium of leading experts to guide a pathway to a cure," while also overseeing funding to external research organizations. Genetic Alliance Inc. develops registries and biobanks for PAOs that choose to partner with it.

E. Other insights

Sections III.A through III.D highlight findings from the 21 variables systematically recorded in the PAO Funding Engagement Dataset. This sub-section highlights other insights gleaned from reviewing PAOs' websites and tax documentation beyond those quantified in the dataset.

The ways in which PAO funders supported research varied widely. Many organizations administered a small number of grants, particularly those more focused on advocacy or providing services to patients. Others oversaw numerous grants for different research projects, often spread across multiple institutions. The extent to which PAO funders were involved in research also varied. A few PAOs, such as the Side Out Foundation, describe having PAO staff serve as project managers throughout the life cycle of a funded study to help keep funded studies on track and coordinate with other funded works.

Many PAOs did not highlight their funding arrangements with life sciences companies on their websites. For these PAO funders, we had to rely on grant lists in PAOs' tax documentation to see their financial arrangements with life sciences companies. In many cases, it was unclear without further investigation that the funding recipient was a life sciences company rather than an academic or other

[7] This count includes PAO funders that conduct most or all of their medical product development activities in house, such as PAOs that do not routinely provide funding to external organizations for research and those that spend less than 50 percent of research expenditures on external partners.

nonprofit institution. In contrast, PAO websites often prominently mentioned funding provided to academic institutions. PAOs might be sensitive to public perceptions of ties to life sciences companies and thus omit references to this funding on their websites. The exception was PAOs that highlighted their venture philanthropy arrangements with life sciences companies on their sites, such as the Boomer Esiason Foundation or the Cystic Fibrosis Foundation. Often, such PAOs have had noteworthy success in discovering and bringing new medical products to market.

One common trend among PAO funders was a focus on young investigator and early-career grants. These grants are intended to help young and/or early-career researchers develop expertise in the hopes they will continue to study a given medical condition and make further research advancements later. Young investigator grants are usually awarded for basic science or preclinical studies. PAOs that award these types of grants had no distinguishing characteristics in terms of size or disease type. For example, Uplifting Athletes awards young investigator grants for research on rare diseases, but PAOs focusing on more common diseases, such as the Breast Cancer Alliance and National Kidney Foundation, also administer such grants.

Many PAOs described efforts to lead and shape medical product development for their condition of focus beyond providing funding (Exhibit 16). Many PAO funders described collaborating with external partners, including life sciences companies, to help with research by consulting on study designs, recruiting patients for clinical trials, or otherwise partnering on research. Other PAOs, such as the Rare Cancer Research Foundation and Pten Hamartoma Tumor Syndrome Foundation, develop and share research tools, such as mouse models, cell lines, and tissue samples, with external researchers to build efficiencies across research teams. Many PAOs, such as the Marfan Foundation, host conferences or organize research collaboratives to enable researchers and clinicians to share findings and advance new areas of exploration for their medical condition of focus. As noted above, several PAOs began operating their own in-house research labs to supplement the research that they fund with external partners or described having in-house research staff co-lead studies with external partners, as is the case with the Side Out Foundation. In conducting these activities, these PAOs serve as leaders in research, influencing the research agenda for their condition of focus.

Exhibit 16. Other strategies PAO funders use to lead medical product development

PAOs highlighted several ways that they lead medical product development activities for their communities beyond funding research and development:

- **Convening researchers** through scientific summits, conferences, and research collaboratives
- **Creating a medical product pipeline** and tracking progress through the research stages
- **Developing preclinical tools** for researchers to use, such as cell lines and animal models
- **Disseminating a strategic plan and priorities** for the community
- **Recruiting patients and engaging clinical networks** to support clinical trials
- **Consulting on research design and outcomes** and helping researchers incorporate the patient voice in new studies ◢

Multiple PAOs reported on their websites that they had stopped funding research grants during the COVID-19 pandemic. For example, the Clear Cell Sarcoma Foundation's website said the PAO stopped funding research other than the patient registry due to the COVID-19 pandemic and the

challenges of raising sufficient funds. Many PAOs saw drops in revenue during the pandemic because they could no longer hold in-person fundraising events and run programs, which likely curbed their ability to fund medical product development activities (IQVIA 2023).

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IV. Discussion

Our study findings highlight the extensive involvement of PAOs in advancing medical product development, from funding basic science research to clinical trials. Other studies have highlighted the changing role of PAOs; although they originated as advocates for patient communities, PAOs are now leaders of innovation in a complex, multistakeholder ecosystem (IQVIA 2021; Reichel et al. 2025). Our findings further quantify this trend. Of the PAOs that we included in our study, 585 PAOs reported funding medical product development activities, comprising more than one-third of all PAOs identified. Of these 585 PAOs, 35 percent reported funding clinical trials, which typically require substantial financial resources. PAOs' financial contributions to medical product research and development have led to noteworthy successes in bringing life-saving drugs to market, in part because PAOs have a uniquely patient-centered approach and are highly motivated to drive innovation (Reichel et al. 2025).

PAOs of all sizes reported funding medical product development activities, but smaller and newer PAOs might face additional challenges in doing so. Although many PAO funders are large and well known, one in four PAO funders are relatively small, with less than $500,000 in annual revenue. Another one in four PAO funders received tax exempt status between 2010 and 2024, suggesting that they are more newly established. Some of these PAOs benefit from member organizations like NORD, which offer guidance to smaller, rare disease PAOs to help them establish medical and scientific advisory boards, create patient registries, and partner with a variety of stakeholders to advance research priorities. However, PAOs that reported partnering with life sciences companies are generally larger, well-resourced PAOs. Only 12 percent of PAOs that fund life sciences companies (12 of 102 PAOs) had an annual revenue of less than $500,000 compared to 25 percent of PAO funders overall. A possible explanation is that PAOs with fewer resources might face additional barriers to partnering with life sciences companies, such as insufficient funding to attract a life sciences partner or lack of clarity about how to initiate a research partnership with life sciences companies (Patterson et al. 2023).

More broadly, relatively limited data were available about PAOs' partnerships with life sciences companies. Although most PAOs were forthcoming with information on their websites about providing financial support to nonprofit partners, such as academic and medical institutions and other PAOs, PAO websites generally did not showcase funding provided to life sciences partners. This was a limitation of the study, in that it hindered our ability to identify all PAOs that fund life sciences companies and understand the amount of funding provided to them. This lack of transparency also poses a challenge to PAOs that are interested in engaging life sciences partners to fund medical product development, making it harder for them to identify other PAOs that have done this work and to learn from them. Although the number of resources describing *how* PAOs and life sciences companies can successfully partner on research is increasing (Reichel et al. 2025; PALADIN n.d.), information is limited about how to initiate and structure these funding arrangements.

Given the successes PAOs have experienced in advancing medical product development by providing financial support for these activities, it is important to consider ways to engage more PAOs in this work and help PAOs sustain these efforts—especially for PAOs that are relatively small and less well established. One way to do this is by developing tools, templates, and peer learning

opportunities for PAOs of all sizes to share how they have initiated successful research partnerships. This could also help spur collaboration and innovation across PAOs. Furthermore, industry and federal government partners could consider ways to make it easier and more transparent for PAOs to engage in partnerships to advance medical product development. For example, PAOs have noted that initiating collaborative efforts can be challenging due to confusion about who to contact and whether they would be open to partnership (Global Genes 2015). Stakeholders could help clarify this information on their websites and create more formal channels for PAOs, academic researchers, and industry partners to establish connections. Another option is to help PAOs consider approaches to increase their revenue. For example, some PAOs have begun monetizing patient registries and data assets as a revenue stream to support and sustain efforts to fund medical product development. However, several of these PAOs have voiced challenges understanding the market and developing such approaches, as well as concerns about conflicts of interest (for example, prioritizing revenue from registry data over innovation and the PAO's mission) and data infrastructure (IQVIA 2021). There might be opportunities to help bring transparency to this process and for PAOs that have grappled with this to share lessons learned and considerations.

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Appendix A

Literature Review Methodology

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We used three approaches to identify relevant literature, which we organized in EndNote:

1. **Searched Google Scholar for peer-reviewed journal articles**. We used the following search string: *"patient advocacy", and "venture philanthropy" or "funded clinical research"*. The date range for the search included all literature published on or before May 28, 2024.[8]This search yielded 157 results, which we further restricted to 25 articles by reviewing abstracts and excluding publications based on the following exclusion criteria:
   - *Publications that do not focus on PAOs' efforts to fund drug development activities* (for example, articles that briefly mention that PAOs may fund medical product development activities but do not include findings related to this topic – this tended to be true of articles that were focused on patient focused drug development and the value of the patient perspective).
   - *Publications flagged as academic theses* (not in peer-reviewed journals). We scanned these publications and saved them for future reference, if needed, but did not extract information from them.
   - *Books*. We skimmed relevant chapters and save them for future reference, if needed, but did not conduct a detailed review.
   - *Publications* that are not available in English.
   - *Publications that were behind paywalls*. We identified nine publications that were potentially relevant but were behind paywalls. We are pursuing options for accessing these articles to inform future work. Most of these articles are six or more years older and were not heavily cited in the relevant recent literature that we reviewed.
2. **Conducted targeted searches on resource pages of prominent organizations.** In an effort to identify grey literature not returned in the Google Scholar search, we searched the resource pages of the following organizations' websites: Kaiser Family Foundation Health News, the U.S. Food and Drug Administration, the Milken Institute and its FasterCures center, and Tufts Patient Advocacy Leaders and Drug Development Industry Network (PALADIN). We identified these organizations – which are involved in research, technical assistance, or policymaking related to PAO-funded drug development activities – through discussions with the Assistant Secretary for Planning and Evaluation and subject matter experts Carolina Reyes, PhD (Illumina Inc.), Jennifer Mills, PhD, MSW, MPH (Foundation Medicine), and Brian Tomlinson, MPA (Foundation Medicine). The search strings varied slightly by website but included some combination of: "patient advocacy," "drug development," "clinical research," and "venture philanthropy." After reviewing the returned literature and applying the exclusion criteria, we identified four additional pieces of literature to include in our extraction efforts.
3. **Additional Google searches.** We conducted broad searches in Google for "patient advocacy, and drug development or clinical research" and "venture philanthropy, and drug development or clinical research" to skim for relevant grey literature that was not uncovered in previous searches. These searches did not yield additional relevant literature beyond the publications identified in the searches described above.

[8] We did not specify a lower date range because we were interested in the evolution of research on this topic. All but two publications returned in the search were published since 2004.

After we identified and organized relevant literature in EndNote, we extracted key information from the final subset of 29 articles in an Excel matrix.

Appendix B

Variable List for PAO Funding Engagement Dataset

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Exhibit B.1. Description of variables in the PAO Funding Engagement dataset

PAO Characteristic	Variable Name	Values	Notes and limitations	Data source
EIN	EIN	9-digit number		Form 990
PAO name	PAO_name	Open-ended text		Form 990
First year as a tax exempt organization	Year_exempt	YYYY		KFN dataset; Form 990
Website address	website	Open-ended text		Form 990; web searches (if missing on Form 990)
Mission statement	Mission_statement	Open-ended text		KFN dataset; Form 990
Total revenue	revenue_amt	Dollar amount of total revenue in the most recent year available [0 – 999,999,999,999]		Form 990
	revenue_year	YYYYMM (e.g., 202212) representing the fiscal year end date associated with revenue_amt		Form 990
Medical product development research activities funded by the PAO	Activities_basic_science	1 = PAO reports funding basic science research to advance medical product development, including natural history studies, research to identify biomarkers, and other basic science research efforts 0 =Unclear or not reported if PAO funds basic science research to advance medical product development	For all research activity indicators, code as 1 if PAO has funded the research activity through financial arrangements with partners or by conducting research in-house, or if they state that the specified research activities are eligible for funding. Information on funded research activities is not reported systematically by PAOs. Some PAOs may not report this information and others may include it in an unexpected location on their website. As such, the research activity indicators may underestimate the number of PAOs engaging in each of these research activities.	PAO website (for example, the 'Research", 'Funded Research', or 'Our research strategy' pages); Form 990, including Part III and Section I

PAO Characteristic	Variable Name	Values	Notes and limitations	Data source
Medical product development research activities funded by the PAO (*continued*)	Activities_preclinical	1 = PAO reports funding pre-clinical research, including *in vivo* or *in vitro* studies, animal models, and high throughput screening 0 = Unclear or not reported if PAO funds pre-clinical research		
	Activities_clinical_trials	1 = PAO reports funding clinical trials 0 = Unclear or not reported if PAO funds clinical trials		
	Activities_registrybiobank	1= PAO reports funding development of a biobank or patient registry or developing one in-house 0 = Unclear or not reported if PAO funds biobank or patient registry development		
	Activities_notspecother	1 = PAO funds medical product development but the funded activities do not fall into the categories above or otherwise cannot be disaggregated (only use if other activity indicators = .) For example, code as 1 if the PAO notes that they have invested money in drug development research, but do not specify the funded research activities. 0 = At least one other development activity indicator equals 1		

PAO Characteristic	Variable Name	Values	Notes and limitations	Data source
Funding invested in medical product development activities	Research_funded__openended	Open-ended field to enter the PAO's reported investments in medical product development research and the relevant timeframe for the investments. If medical product development research investments are not available, total research investments may be recorded. The data source for the funding amount is included in parenthesis. . = Unclear or not reported	PAOs do not systematically report this information. Entries may reflect funding amounts in a given year, since a PAO's inception, or another interval. In cases where PAOs do not report medical product development investments, (e.g., grants flagged as "drug research" in Form 990), the field reflects overall research investments, including research unrelated to medical product development. We denote this in the open-ended field. As such, values reported in this field may overestimate medical product development research funding.	PAO website (for example, the 'Impact" or "Research" pages or Annual Impact Reports); Form 990, including Part III and Section I
	Annual_research_funded_estimate	Annual research investments in the most recent year reported. For PAOs that cite investments in research over a multi-year period, we estimate the average annual funding amount by dividing total research investments by years funding research. For example, $50,000,000 over 5 years of operation = $10,000,000 per year. If calculating the annual amount based on funded amount since inception, use end of 2023 for calculations unless otherwise stipulated (for example, if the website says "from inception through 2022"). . = Unclear or not reported	PAOs do not fund research in equal increments each year. Estimated annual research amounts are intended to inform the categorical variable (*annual-research_funded_categorical*) and are unlikely accurately depict a PAO's investments in a given year.	

PAO Characteristic	Variable Name	Values	Notes and limitations	Data source
Funding invested in medical product development activities (*continued*)	Annual_research_funded_categorical	1 = <$100,000 per year = Most recent or estimated annual amount of research funding is less than $100,000 2 = $100,000 to $999,999 per year = Most recent or estimated annual amount of research funding is between $50,000 and $999,999 3 – Over $1,000,000 = Most recent or estimated annual amount of research funding is over $1,000,000 . = Unclear or not reported how much PAO invests in medical product development research/ research	This field is based on estimated research amounts in the *annual_reserach_funded _estimate* field, so may not accurately reflect a PAO's research investments.	
Types of funding arrangements with medical product development partners	FA_in_house_research	1 = PAO conducts basic science research activities in-house (e.g. PAOs that maintain patient registries or biobanks in-house or have in-house staff on payroll who conduct basic science research). 0 = Unclear or not reported whether PAO conducts basic science or preclinical research activities in-house	Additional details on types of arrangements (for example, if the PAO uses a venture philanthropy model or operates as a non-profit biotech firm) are included in the Notes column.	Form 990, including Part III and Section I; PAO websites (for example, the 'Our Research Strategy' page or list of past grantees)
	FA_in_house_trials	1 = PAO staff lead clinical trials for new drugs 0 = Unclear or not reported whether PAO staff lead clinical trials for new drugs		

PAO Characteristic	Variable Name	Values	Notes and limitations	Data source
Types of funding arrangements with medical product development partners (*continued*)	FA_in_house_biotech	1 = PAO primarily operates as a non-profit biotech organization that develops new therapeutics in-house. This includes PAOs that conduct *all or most* medical product development activities in-house (i.e., PAOs that do not routinely provide funding to external organizations for research and those that spend less than 50% of research expenditures on external partners). 0 = Unclear or not reported whether PAO primarily operates as a non-profit biotech organization that develops new therapeutics in-house		
	FA_in_house_other	1 = PAO has another type of arrangement where in-house staff have substantial involvement in medical product development activities (e.g., PAO leads a research consortium or PAO is a membership organization that supports other PAOs' efforts to engage in drug development activities) 0 = Unclear or not reported whether PAO has some other type of arrangement where in-house staff have substantial involvement in drug development research activities		
	FA_grants_academic_med	1= PAO funds academic or medical research institutions for MPD research/ development 0 = Unclear or not reported whether PAO funds grants to academic or medical research institutions		

PAO Characteristic	Variable Name	Values	Notes and limitations	Data source
Types of funding arrangements with medical product development partners (*continued*)	FA_to_industry	1 = PAO provides funding directly to industry partners (i.e., life science organizations, including pharmaceutical companies for MPD research/ development (either through grants, venture philanthropy, or other arrangements) 0 = Unclear or not reported whether PAO provides funding to industry partners		

PAO Characteristic	Variable Name	Values	Notes and limitations	Data source
Efforts to lead or shape medical product development	Med_Prd_Dev_Leadership_Notes	Free-text field that describes ways that PAOs report leading or shaping medical product development activities for their respective disease communities. Types of activities highlighted in this field include: • Employing an internal research team to conducts PAO-initiated research • Fostering collaboration among the research community by organizing research conferences or consortia • Providing expertise or guidance to the research community through support for design or analysis, or provision of research tools (e.g., cell lines, tissue samples, mouse models) • Maintaining a drug pipeline on the PAO website • Sharing a strategic plan for advancing medical product development activities on the PAO website • Highlighting the PAO's role as a leader in medical product development on the PAO website (e.g., by listing discoveries and achievements to date) • Describing other ways the PAO leads medical product development activities on the website . = Not populated because PAO funds an average of $0 to$999,999 in research annually (Annual_research_funded_categorical= 1 or 2)	Field is only populated if PAO funds an average of $1,000,000 in research annually or research funding is unknown (Annual_research_funded_categorical = 3 or .).	PAO websites
Additional details about PAOs	Notes	Free-text field to capture additional notes about PAOs' funded activities, investments, or funding arrangements.		N/A

KFN = Kaiser Family Network; PAO = Patient Advocacy Organization.

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