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Short Communication

Environmental scan of cystic fibrosis research worldwide

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Abstract

Background: Cystic fibrosis (CF) is a rare fatal genetic disease, affecting 70,000 to 100,000 people worldwide [1]. Numerous countries have specific charitable organizations dedicated to CF, with many funding research to find a cure or effective control for the disease. Cystic Fibrosis Canada, the largest charity in Canada dedicated to funding research and care in CF, conducted an environmental scan of these organizations to learn and better understand their research goals and funding process.
Methods: A set of questions was sent to 25 CF charitable organizations around the world by email. Responses were consolidated to identify common practices and innovative approaches.
Results: Among respondents, there were variations in the amount of funds invested in research annually and the number of studies supported. Common themes identified included practicing an open call for research applications, evaluating applications using a peer review process, and placing an increased emphasis on patient engagement. Innovative approaches included funding one larger project; funding a series of sub-projects on a common theme; partially funding a research project; and, indefinitely funding part of a researcher's salary.
Conclusions: Among CF charitable organizations, there are numerous approaches to research funding. Both similarities and differences were noted between these organizations, all of which share the common goal of working towards improving quality of life and survival for people with CF.

Keywords: Cystic fibrosis; Rare disease; Charitable organizations; Research funding; Environmental scan

1. Introduction

Cystic fibrosis (CF) is a rare fatal genetic disease affecting 70,000 to 100,000 people worldwide, including over 4100 people in Canada [1]. CF charitable organizations exist in over 40 countries; 15 of these organizations are known to fund CF research. This research funding is essential and has led to considerable advances in scientific understanding of the disease, including the gene discovery in 1989. There are countries with substantial CF populations who lack CF charities that support research, such as South Africa and Israel (CF populations of approximately 700 and 650 respectively); however, these countries can still benefit from research happening elsewhere in the global scientific community. CF charitable organizations that do not fund research typically choose to focus on supporting individuals and families with CF directly, such as by providing access to equipment and medications, rather than investing in research.

In spring 2016, Cystic Fibrosis Canada conducted an environmental scan of CF research funded by CF charitable organizations to gain a global picture of active research in terms of dollars spent and topics studied. An understanding of current practices will better enable CF charitable organizations to work together as a global community.

2. Materials & methods

Cystic Fibrosis Canada contacted 25 CF charitable organizations around the world from the countries shown in Fig. 1. Organizations were identified through Cystic Fibrosis Worldwide's website (cfww.org) [1]. The purpose of the environmental scan was to focus on research; therefore, countries with CF organizations that are known not to fund research were excluded from the scan.

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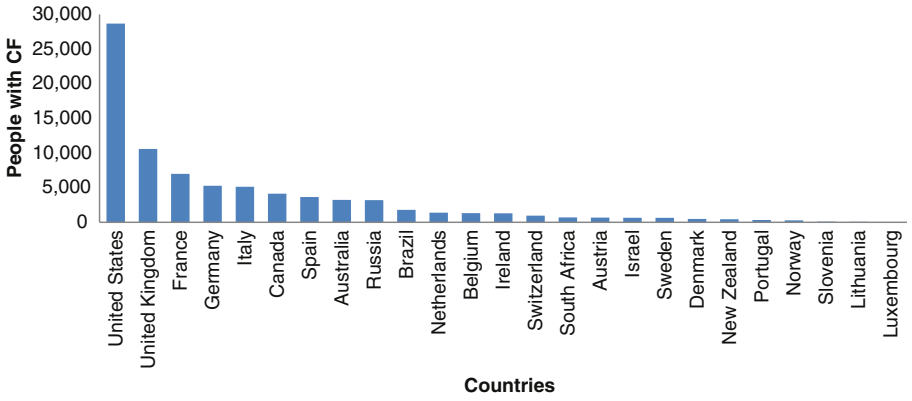

Fig. 1. CF population in countries with CF charitable organizations contacted for the environmental scan. For data sources, see Appendix A.

3. Results

Responses were received from 14 CF charitable organizations (56% response rate): Cystic Fibrosis Australia (AU); Association Muco (BE); Cystic Fibrosis Canada (CA); Cystisk Fibrose Foreningen (DK); Vaincre la Mucoviscidose (FR); Mukoviszidose (DE); Cystic Fibrosis Foundation of Israel (IL); Fondazione Ricerca Fibrosi Cistica – Onlus (IT); Nederlandse Cystic Fibrosis Stichting (NL); All-Russian Association for Cystic Fibrosis Patients (RU); Riksforbundet Cystisk Fibros (SE); Cystic Fibrosis association Switzerland (CH); Cystic Fibrosis Trust (UK); and, Cystic Fibrosis Foundation (US). Each organization was asked a series of questions about their research and research funding process. For a table summary of results, see Tables 1 and 2. Cystic Fibrosis Foundation of Israel (IL) and All-Russian Association for Cystic Fibrosis Patients (RU) do not fund research; therefore, their responses are not included below.

4. Discussion

Three common themes across organizations were observed: practicing open calls; peer review; and patient engagement. Distinct innovative approaches were noted.

4.1. Open call

Almost all CF organizations practiced open calls for research applications, meaning that researchers focusing on any type of research related to CF are welcome to apply for funding. This is in contrast to targeted calls, which invite researchers to put forward applications for funding in specific areas, such as gene therapy. While the majority of CF charitable organizations exclusively practice open calls, a select number practice a mix of open and targeted calls. For example, Cystic Fibrosis Foundation (US) funds the majority of their basic science research through open calls; however, in 2015 they targeted gene editing, stem cells and gene delivery in a strategic move to fund projects to benefit the last 5% of patients that may not be eligible for CFTR modulators. In 2016, they targeted transplant immunology.

4.2. Peer review

All CF organizations rely on a peer review process to evaluate research applications and make funding decisions. Research applications are sent out for review to experts in the field of the particular application subject; many countries use international experts to review applications if they lack a particular expertise within their own country. Applications are evaluated by anywhere from two to six reviewers, with some reviewers providing a critique and scoring, and others providing solely a critique, along with a Yes/No recommendation for funding.

4.3. Patient engagement

Patient engagement is a priority across CF organizations, with 10 CF organizations including patient representatives on

Table 1
Summary table of CF charitable organizations' responses.

Question	CF charitable organization (identified by country)											
	AU	BE	CA	DK	FR	DE	IT	NL	SE	CH	UK	US
Open call		✓	✓	✓	✓	✓	✓		✓	✓	✓	✓
Mix of open call and targeted											✓	✓
Peer review	✓	✓	✓	✓	✓	✓	✓	✓	✓	✓	✓	✓
Funding restrictions to researchers in own country	✓	✓	✓	✓	✓	✓			✓	✓	✓	
Amount invested annually (USD)[a]	$.424M	$.333M	$3.87M	$.087M	$3.0M	$.554M	$2.5M	$.554M	$.041M	$.050M	$4.9M	$44M[b]
Number of studies per year (2016)	1	6	52	3	70	5	25	4	3	3	3	265
Patient engagement		✓	✓	✓	✓	✓	✓	✓	✓	✓	✓	

[a] Approximations based on rounding to the nearest major dollar amount.
[b] Basic science research investments.

Table 2
Main research project topics.

CF charitable organization	Main topics
Cystic Fibrosis (AU)	Infection (multi-action antibiotics)
Association Muco (BE)	Infection, quality of life (lung treatments, healthcare accessibility), organoids
Cystic Fibrosis Canada (CA)	Infection, quality of life, CFTR structure and function
Cystisk Fibrose Foreningen (DK)	Improvement and optimizing treatment of CF patients at the two Danish CF Centers
Vaincre la Mucoviscidose (FR)	Infection, inflammation, CFTR modulators
Mukoviszidose (DE)	CFTR activation, inflammation, microbiology, diagnostic methods (lung imaging, biomarkers, quality of life)
Cystic Fibrosis Ireland (IE) *	Quality of life (depression and anxiety)
Fondazione Ricerca Fibrosi Cistica – Onlus (IT)	CFTR modulators, infection, inflammation
Nederlandse Cystic Fibrosis Stichting (NL)	Precision medicine, organoids
Riksforbundet Cystisk Fibros (SE)	Improving CF treatment
Cystic Fibrosis association Switzerland (CH)	Inflammation, quality of life
Cystic Fibrosis Trust (UK)	CFTR modulators, gene therapy, stem cells, infection, inflammation
Cystic Fibrosis Foundation (US)	CFTR modulation, infection, inflammation, gene editing, stem cells, gene delivery

* Cystic Fibrosis Ireland (IE) did not respond; this information was gained from their website.

their scientific and/or executive boards, meaning that patients contribute to deciding which projects to fund.

4.4. Innovative approaches

Approaches practiced by select organizations that stood out included: funding one larger project, rather than 3–4 smaller projects (Cystic Fibrosis Australia (AU)); funding one larger project with a series of sub-projects focused on a common theme (Nederlandse Cystic Fibrosis Stichting (NL)); partially funding a research project (Riksforbundet Cystisk Fibros (SE) and Cystic Fibrosis Foundation (US)); and, indefinitely funding a researcher's salary (Riksforbundet Cystisk Fibros (SE)).

5. Summary

The environmental scan was well received by CF charitable organizations around the world, with all responding organizations indicating support for the project and a desire to collaborate. The scan revealed common practices and innovative approaches. Unfortunately, it was out of the scope of the current project to examine the impact of CF charitable research per dollar (i.e., it is difficult to measure the success of various funding practices). Despite this limitation, the scan does provide insight into CF charitable research, with implications for knowledge-sharing across organizations.

6. Conclusion

In conclusion, this environmental scan identified 15 countries with CF organizations that fund research. The scan increased understanding of this research, which is necessary to ensure that all areas of potential investigation are covered and that CF charitable organizations in different parts of the world are able to learn from each other.

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Appendix A

Total country populations, cystic fibrosis populations and cystic fibrosis population percentages:

Country	Population	CF Population	Percentage of population	Source
Australia	24,309,330	3235	0.013%	2013 CF AU registry
Belgium	11,371,928	1318	0.012%	2013 ECFS registry [a]
Brazil	209,333,250	1798	0.000%	2010 BR registry
Canada	36,234,552	4128	0.011%	2014 CF CA registry
Denmark	5,690,750	481	0.008%	2013 ECFS registry
France	64,668,129	6984	0.011%	2013 ECFS registry
Germany	80,682,351	5266	0.007%	2010 ECFS registry
Ireland	4,713,993	1299	0.028%	2013 ECFS registry
Israel	8,192,463	649	0.008%	2013 ECFS registry
Italy	59,801,004	5130	0.009%	2013 ECFS registry
Lithuania	2,851,704	65	0.002%	2013 ECFS registry

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Appendix A (*continued*)

Country	Population	CF Population	Percentage of population	Source
Luxembourg	578,674	20	0.003%	2005 CFF annual report [b]
The Netherlands	16,979,729	1387	0.008%	2013 ECFS registry
New Zealand	4,565,185	443	0.010%	2014 CF NZ registry
Norway	5,271,958	280	0.005%	2009 CFWW site [c]
Russia	143,439,832	3194	0.002%	2013 ECFS registry
South Africa	54,978,907	700	0.001%	2009 SA CFA site [d]
Spain	46,064,604	3648	0.008%	2013 ECFS registry
Sweden	9,851,852	646	0.007%	2013 ECFS registry
Switzerland	8,385,353	955	0.011%	2013 ECFS registry
United Kingdom	65,049, 933	10,583	0.016%	2014 CFT registry
United States	322,762,018	28,676	0.009%	2014 CFF registry

[a] European Cystic Fibrosis Society.
[b] 2005 Cystic Fibrosis Foundation annual data report to the center directors [2].
[c] Cystic Fibrosis Worldwide website (cfww.org).
[d] South African Cystic Fibrosis Association website (sacfa.org.za).

Appendix B

Cystic Fibrosis organizational contacts:

Country	Organization	Website	Contact name	Position
Australia	Cystic Fibrosis Australia	cysticfibrosis.org.au	Nettie Burke; Graham Gourlay	CEO; Special Projects
Belgium	Association Muco	muco.be	Ulrike Pypops	Deputy Director
Canada	Cystic Fibrosis Canada	cysticfibrosis.ca	Joanna Valsamis	Chief Officer, Research, Advocacy and Healthcare
Denmark	Cystisk Fibrose Foreningen	cystiskfibrose.dk	Helle Ousted	Director
France	Vaincre la Mucoviscidose	vaincrelamuco.org	Paola De Carli; Véronique Marguerite-Heissat	Scientific Director; Deputy Scientific Director
Germany	Mukoviszidose	muko.info	Sylvia Hafkemeyer	Scientific Manager
Israel	Cystic Fibrosis Foundation of Israel	cff.org.il	Shira Zagury	General Director
Italy	Fondazione Ricerca Fibrosi Cistica - Onlus	fibrosicisticaricerca.it	Gianni Mastella	Scientific Director
The Netherlands	Nederlandse Cystic Fibrosis Stichting	ncfs.nl	Vincent A.M. Gulmans	Research Coordinator
Russia	All-Russian Association for Cystic Fibrosis Patients	mukoviscidoz.org	Nataliya Kashirskaya	Professor, Dept. of genetic epidemiology (Cystic Fibrosis group)
Sweden	Riksforbundet Cystisk Fibros	rfcf.se	Ludvig Arbin	Executive Director
Switzerland	Cystic Fibrosis association Switzerland	cfch.ch	Sara Hofmann	Manager
United Kingdom	Cystic Fibrosis Trust	cftrust.org.uk	Janet Allen	Director of Research
United States	Cystic Fibrosis Foundation	cff.org	Katherine L. Tuggle	Director of Research

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