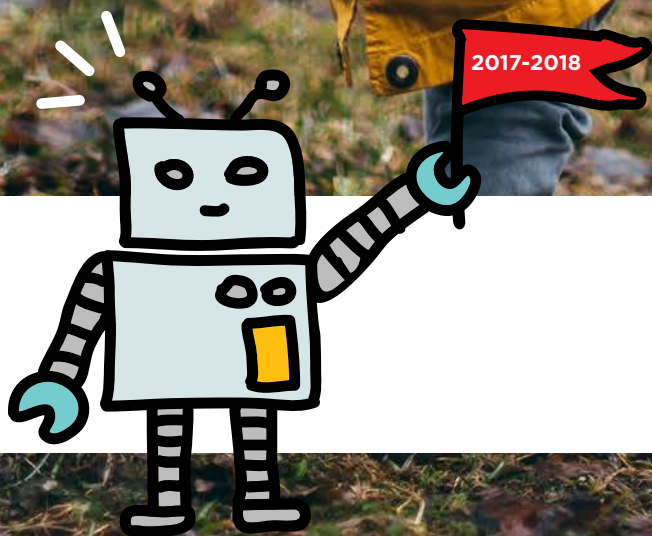


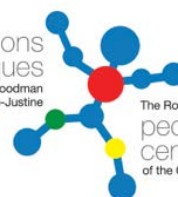


ANNUAL REPORT

2017-2018

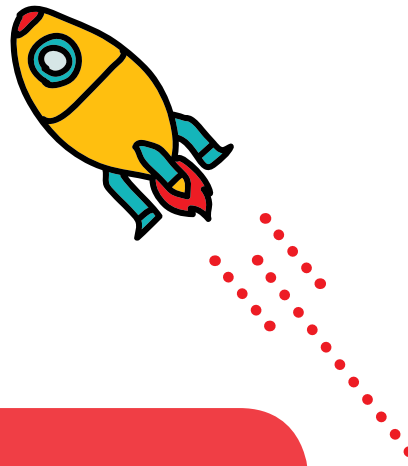


centre de formulations
pédiatriques
de la famille Rosalind et Morris Goodman
du CHU Sainte-Justine



The Rosalind & Morris Goodman Family
pediatric formulations
centre
of the CHU Sainte-Justine

TABLE OF CONTENTS



3
Directors' Message

4
The Goodman
Pediatric
Formulations
Centre

5
Goals of the Centre

6
Services Offered

7
Achievements
2017-2018

11
Acknowledgments

12
Addendum

12
List of acronyms

12
Contact Person



Catherine Litalien



Andrea Gilpin

DIRECTORS' MESSAGE

Over the past year, the Goodman Pediatric Formulations Centre (GPFC) has come a long way, taking part in many local and international meetings, at which some team members were recognized as leaders in facilitating access to pediatric formulations. An important part in advocating for pediatric medications is to understand which medications are most in need of the development of a pediatric formulation. To this end, a prioritized list was created of drugs requiring new formulations appropriate for children. This priority list was the result of a 2017 survey addressed to pharmacists representing 13 pediatric hospital centres across the country. The list was presented at several international conferences including, EuPFI¹, AAPS, and ASCPT. With this priority list in hand, the GPFC sought partners in the pharmaceutical industry in order to raise their interest in eventually bringing the listed drugs into Canada.

The GPFC continues to put its expertise to work raising certain key players' awareness of issues related to pediatric formulations, as well as participating in the creation of an atmosphere that is favourable to the development of pharmaceutical forms specially designed for children. Many discussions were held in the last few months with government authorities, among which were Health Canada, the INESSS, and the CADTH. Rallying these government agencies is the first step in transforming and improving the present health care system. It is also of the highest importance to engage with politicians, pediatricians, pharmacists, other Canadian (and foreign) pediatric hospitals, the pharmaceutical industry, as well as patients and their families who are the heart of our project.

In conclusion, we hope that the year 2018-2019 will bring closer the arrival of new drugs adapted to our young Canadian patients. This is the whole meaning of our mission. We invite you to visit our website, share this Annual Report with colleagues and friends, and follow us on social media in order to keep abreast of our latest news.

A stylized black ink signature of Catherine Litalien.

Catherine Litalien
Executive Director

A stylized black ink signature of Andrea Gilpin.

Andrea Gilpin
General Manager

1. The list of acronyms can be found on page 12 of this document.

THE GOODMAN PEDIATRIC FORMULATIONS CENTRE

Who We Are

The Goodman Pediatric Formulations Centre (GPFC) of the CHU Sainte-Justine is a non-profit and was created in 2016 thanks to the contribution of the Rosalind & Morris Goodman Foundation. The GPFC is committed to improving access to child-friendly medicines by increasing the efficacy, safety and compliance of medicines for children. To achieve these important and ambitious goals, we have brought together pediatricians and pharmacists with the requisite depth of expertise and breath of influence.

Mission

To be the voice of Canadian children, of all ages, in order to improve access to medicines that meet their needs by creating an environment conducive to the development and marketing of pediatric formulations. In order to build a sustainable Centre, the GPFC works around four pillars:

- 1 Operationalize the GPFC
- 2 Promoting Access to Pediatric Formulations
- 3 Recognition of Expertise & Leadership
- 4 Building Advocacy for the Cause

Why do we need a Pediatric Formulations Centre?

Many medicines used in children are not available in pharmaceutical forms adapted to their needs. Indeed, most oral medications are produced for adults as tablets and capsules, which lack dosing flexibility to meet dosage requirements for the spectrum of ages and sizes, ranging from neonates to adolescents. Furthermore, most children under the age of 8 are unable to swallow pills. Approved adult forms often need to be modified in some manner to administer the desired dose to children, and as such are used off-label. When this happens, the adult form is manipulated by either a health care provider, such as pharmacist, or at home by the parents or caregivers (e.g., grinding a tablet and sprinkling it into fluid or a food vehicle such as apple sauce). This process is called compounding and is commonplace for those medicines that lack pediatric formulations. [Click here to watch a short video](#). Given that compounded medicines are not approved by Health Canada's rigorous process, we do not always know the medication's characteristics. Therefore, it is sometimes difficult to know the exact quantity of the medicine that is absorbed. Furthermore, the food vehicle (e.g., apple sauce, juice or yogurt) used to administer these compounded medicines, or to mask the bad taste of some formulations, can alter drug absorption. We strive to contribute to making available convenient and high-quality pediatric products adapted to children's needs. It is important as the formulation can spell the difference between successful treatment and therapeutic failure as well as reduce side effects.

GOALS OF THE CENTRE

1. To facilitate the development and market authorization of safe and effective pediatric drug formulations by:

- Promoting a research-based approach to pediatric formulation development;
- Contributing to a clinical and regulatory environment that fosters pediatric formulation development;
- Contributing to uncovering incentives that could stimulate and attract the development of pediatric formulations;
- Identifying hurdles and challenges in the drug development process as well as in the regulatory landscape and act as a change agent;
- Promoting cost effective treatment for children.

2. To promote safety of medicines administered to children.

These goals are aligned with a 2012 report of the Council of Canadian Academies:

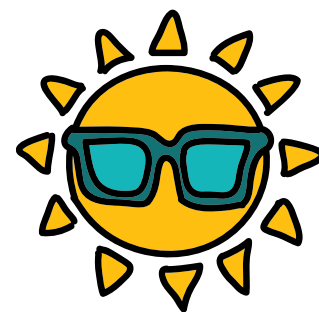
“Improving Medicines for Children in Canada”



In addition, our mandate supports equal access to pediatric medicines across the country which is aligned with a recent report of the [Pan-Canadian Health Organizations](#) that cited equality as an important attribute to our health care system.



SERVICES OFFERED



The GPFC provides to the pharmaceutical industry, or other Parties, the following services in relation with the development and regulatory approval of pediatric formulations:

1

Clinical, pharmacological and regulatory expertise

- Identification and prioritization of children's needs
- Development of Pediatric Formulation Development Plan (PERFORM)
- Writing of a Mini-Perform called PMCA (Pediatric Medical and Clinical Assessment) which includes the size of the Canadian pediatric market in order to provide data on certain drugs to the pharmaceutical partners, thus helping industry to create a business plan
- Writing of a systematic review using the Cochrane methodology
- Consultation services (clinic, business, regulatory, and other)

2

Clinical research services and expertise

- Research capacity with clinical investigation unit and trained personnel in pediatrics
- Expertise in the design and conduct of pediatric pharmacokinetic, efficacy and safety studies

**Additional services may be provided, as needed.*

ACHIEVEMENTS 2017-2018



1

Operationalize the GPFC

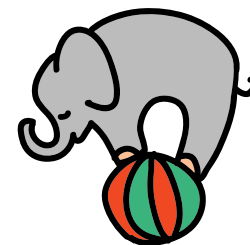
- Goal:** To continue to build the Centre and develop a highly functioning team to serve the GPFC's mission and purpose.
- Strategy:** Contribute to the synchronization and the efficiency of the Centre's team; diversify the Centre's service offerings according to the needs of our partners.

Our Accomplishments

- The GPFC launched its own website in September 2017
- GPFC materials were created: a conference banner, infographic and a social media presence ([Facebook](#), [Twitter](#), and [LinkedIn](#)) since March 2018.
- The GPFC begun an internal communications effort at both the CHU Sainte-Justine Hospital and its Research Centre:
 - a. The GPFC participated with one half day of presentations for “*La semaine de la Qualité et de l'Innovation*” of the CHU Sainte-Justine where three presentations were made regarding pediatric formulations, two of which were external guest speakers
 - b. The GPFC presented at the Medical Grand Rounds where healthcare professionals gather monthly to learn more about new subjects of interest
 - c. The GPFC appeared in the monthly *Infolettre* of the CHU Sainte-Justine Research Centre in March 2018
 - d. Appears now on the CHU Sainte-Justine website under the thumbnail [Research - Platforms and services - Platforms](#)
- The GPFC has undergone a reorganization in order to align with its strategy. As a result, two positions were eliminated, and two others were created: the position of Operations and Communications Advisor and the position of Scientific and Medical Lead who is also a pharmacist with pharmaceutical industry experience. In addition, the GPFC has support to managing our accounting and invoicing who works one day per week.

2

Promoting Access to Pediatric Formulations



- Goal:** Promoting access to commercial pediatric formulations through partnerships with the pharmaceutical industry.
- Strategy:** The first initiative consists of finding pharma partners to develop and promote medicines that the GPFC has identified nationally on its priority list. A second initiative is to contribute to developing incentives to entice pharma investment in pediatric formulations by working with key stakeholders.

Our Accomplishments

- Over the last year, exchanges were held with **23 pharmaceutical companies** and **7 confidentiality agreements were signed**. In addition, 7 agreements of the same type were signed with other partners.
- We continue to work with a pharmaceutical partner who has been collaborating with us since the Centre's inception. This partnership led to the submission of a drug application (amlodipine) to Health Canada in March 2018 and the submission of another drug (leviteracetam) will be made in mid 2018.
- Two collaboration agreements are in place with pharmaceutical companies for two drugs on the Centre's list of priority drugs.
 - One of the pharmaceutical partners has already stated its interest in investing time and money with the Centre to develop a formulation adapted to children, that does not exist elsewhere in Canada, in Europe, or in the United States.
 - The second potential partnership we are awaiting the choice of their medication to develop as well as a financing plan for the project. In both of these projects we expect to receive term sheets for negotiation over the summer.
- A list of priority drugs was first conceived by the CHU Sainte-Justine and was later validated with the help of a census of 13 pediatric hospital centres across the country.
 - The list is divided into three categories according to precise criteria
 - Technical data files have been completed for 20 targeted drugs
 - The list of priority drugs was presented to the **DIA** in Ottawa in June 2017, at the international conference of the **EuPFI** in September 2017, to the **AAPS** in November 2017, and to the **ASCT** in March 2018; it elicited lively interest
 - Two publications are in progress:
 - Categorization Study*
 - Pan-Canadian Study*

3

Recognition of Expertise & Leadership



Goal: To build further our expertise in the field and to use this growing knowledge to become an internationally recognized leader in improving access to pediatric formulations. We would like to become the go-to resource for pediatric formulations in Canada and be involved in global initiatives in this area.

Strategy: Increase the profile of the GPFC externally (conferences, meeting stakeholders, position statements, invited to sit on working committees, etc.). Increasing the profile of the Centre within CHU Sainte-Justine and develop internal champions for each therapeutic area is also a key priority under this pillar. In addition, we will contribute, where possible, to increasing the safety of pediatric medicines in children by using our expertise where it is needed.

Our Accomplishments

The GPFC shone at numerous local and national meetings:

- Keynote speaker at the Canadian Meeting of the **DIA** in Ottawa in June 2017
- Keynote speaker, scientific poster and booth at the international **EuPFI** conference in September 2017
- Presenter on international panel, scientific banner, booth and meetings during the reunion and the annual exposition of the **AAPS** in November 2017
- Oral presentation during the annual meeting of the **ASCPT** in March 2018
- Meeting in Quebec with the **Canadian Society of Hospital Pharmacists** to present a scientific banner
- Speaker at the Medical Grand Rounds at the CHU Sainte-Justine on March 28, 2018



Some new partnerships were founded:

- **The Canadian Paediatric Society** and the Goodman Pediatric Formulations Centre organized a joint meeting in January 2018, bringing together 21 participants from 10 different organizations. This was a first in terms of bringing together pediatricians, pharmacists, and experts on reimbursement and regulations. **The Canadian Paediatric Society** and the Goodman Pediatric Formulations Centre are currently drawing up a Policy Paper with the support of the experts and organizations who participated in the meeting. The Policy Paper outlines the challenges and difficulties of bringing pediatric formulations to Canada. It will be presented to Health Canada in the fall 2018 and will be posted on our Website when completed.
- The GPFC is continuing to gain prestige at the CHU Sainte-Justine with internal champions in the gastroenterology, nephrology, cardiology and neurology fields.

4

Building Advocacy for the Cause



Goal: To increase awareness and, using our expertise, contribute to shaping the landscape to improve access to pediatric formulations in Canada.

Strategy: Building on two key reports that were published in the last few years, **“Improving Medicines in Canada”** and **“Prescription Medicines in Canada – Off-Label Use”** we are developing a strong alliance with major stakeholders to contribute to improving the process by which pediatric medicines are approved in Canada.

In addition to meeting with Health Canada, the GPFC is seeking to understand the position and work together with various stakeholders, such as industry associations, to support changes that will attract investment in pediatric formulations in Canada. Acting as a change agent, we will use our team’s expertise (clinical, research, regulatory, etc.) to support initiatives to make the Canadian market a favourable country in which to develop and promote pediatric formulations.

Our Accomplishments

- Since January 2018, preliminary discussions have been underway with the CADTH on the process of reimbursement of pediatric formulations
- The Centre contacted the INESSS’s Drugs Directorate in order to introduce the Centre’s mission and its activities
- A meeting took place with the Université de Montréal’s Institut universitaire de gériatrie to discuss some similarities between children and the elderly insofar as the administration of drugs is concerned
- A strategy targeting reimbursement and the government with respect to generic drugs is being developed
- In partnership with KidsCAN Trials, the GPFC sent a letter³ to Health Canada that did not support the increasing of the fees related to marketing a drug in pediatrics
- A letter of support⁴ was sent following a consultation with Health Canada regarding a possible new regulatory pathway for the authorization of the sale of drugs already approved by trusted foreign regulators. This new potential approach process could facilitate access for Canadians to drugs that meet a healthcare system need
- We took part in a consultation concerning the renewal of Health Canada’s special access program (SAP)
- We took part in a web survey in order to provide comments from a pediatric point of view about changes that could be made to Health Canada’s Priority Review process

3. See the Addendum to read the letter sent to Health Canada

4. See the Addendum to read the letter sent to Health Canada.



ACKNOWLEDGMENTS

We would like to warmly thank the Morris & Rosalind Goodman Family Foundation, the CHU Sainte-Justine and our valued partners. We also thank the people who support us by providing their advice, support and expertise, in kind. A heartfelt thank you to all!



ADDENDUM

[Letter of protest sent to Health Canada](#)

[Letter of support following a consultation by Health Canada](#)

[To see the GPFC's scientific posters, please visit our website](#)

LIST OF ACRONYMS

EuPFI: European Paediatric Formulation Initiative

INESSS: Institut National d'excellence en santé et en services sociaux

CADTH: Canadian Agency for Drugs and Technologies in Health

AAPS: American Association of Pharmaceutical Scientists

ASCPT: American Society for Clinical Pharmacology & Therapeutics

DIA: Drug Information Association

PMCA: Pediatric Medical and Clinical Assessment

PERFORM: Pediatric Formulation Development Plan

CONTACT PERSON

Andrea Gilpin, General Manager

Tel.: 514 345-4931, extension 3655

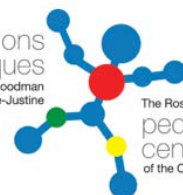
Website: gpfccanada.com





Thanks!

centre de formulations
pédiatriques
de la famille Rosalind et Morris Goodman
du CHU Sainte-Justine



The Rosalind & Morris Goodman Family
pediatric formulations
centre
of the CHU Sainte-Justine