A MESSAGE FROM OUR CO-FOUNDEES

When we founded the RUNX1 Research Program (RRP) in 2016, we could only imagine a community of patients and their families, researchers, clinicians, genetic counselors, and others uniting to build awareness of RUNX1-FPD and to advance research dedicated to improving the quality of life for those living with the disease. With four generations of RUNX1-FPD in our family, there was an urgency to our efforts. We wanted answers and we wanted solutions.

Today it is a reality. Collaboration is at the core of our ethos – we are eager for every program, every grant, every facet of what we do to be about avoiding the traditional silos that have existed in science. By bringing everyone to the table and engaging their expertise, we are able to harness so much more knowledge and power.

We are incredibly proud of the progress and impact RRP has achieved in such a short time. But there is still much to do. So, please take a moment to read this impact report and join us in our pursuit to find a cure and prevent cancer for RUNX1-FPD patients worldwide.

With gratitude,

Tim Babich
Co-Founder & Director

Monica Babich
Co-Founder & Director
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**One goal. Prevent cancer.**

At the RUNX1 Research Program (RRP), our mission is to improve the quality of life and prevent cancer in patients with **RUNX1-FPD**.

We do this by promoting awareness and funding world-class collaborative research with an empowered patient community.
2021 was truly a landmark year for the RUNX1 Research Program (RRP).

Since joining the Foundation over three years ago, I have been deeply involved in all aspects of our work - from overseeing our ever-growing research portfolio to supporting patients and clinicians through a host of educational programming and beyond - and I am amazed at the momentum that has been generated by our entire community in order to advance our mission in a short period.

As we celebrated our five year anniversary, it was a particularly opportune time to reflect upon our accomplishments (see page 3 for our historical timeline) and look to the future.

I am proud to say that we have awarded over $9M in grants supporting 31 projects since 2017. This was an incredible milestone for RRP and due to our investment, we can report on some exciting successes.

Our research community has developed six disease models which will help test future treatments. We also learned that the prevalence of germline RUNX1 mutations in RUNX1-mutated AML is between 10-30%, highlighting that this disorder is not that rare.

We launched two new grant programs, FIRE and STRIVE, dedicated to further investigating whether we can use medicines that control inflammation and/or enhance the immune system to prevent cancer.

Finally, we facilitated the establishment of a cross-institutional, cross-disciplinary working group to draft the design of the first clinical trial study to test an FDA-approved drug called imatinib for the treatment of RUNX1-FPD.

What does this all mean?
It goes back to our main goal - to prevent cancer so families don’t have to lose their loved ones. We cannot ignore that cancer is on track to be the number one cause of death in the United States in a few years.

We push so hard to move cancer prevention research forward as quickly as possible because, unfortunately, the heightened risk of fatal blood cancers for RUNX1-FPD patients means time is never on our side.

Fortunately, the interventions we discover for RUNX1-FPD patient families, may benefit others at risk for cancer too. RUNX1-FPD patients just might hold the key to understanding how the first cancer cell arises and how it can be eliminated before full-blown cancer occurs.

It is a lofty mission, but we are certain that we will get there. If you aren’t already a supporter, please consider joining our efforts as we diligently march toward a cure for those living with RUNX1-FPD.

Here’s to another successful year!
Thank you for your partnership.

[Signature]

RUNX1 Research Program Team

Amanda Eggen, PhD
Director of Patient Engagement & Clinical Programs

Alex Gonzalez
Director of Development

Catherine Pelton
Communications Associate
FIVE YEARS OF SUCCESS

The *RUNX1* Research Program has experienced tremendous growth since its inception. As part of our five year anniversary, we launched a special campaign, *X-Ponential*, that celebrated our achievements.

This timeline commemorates our many accomplishments and illustrates our impact. Here are some of our most recent major milestones.

2021

- **Dr. Lucio Castilla** further characterizes the relevance of his human germline Runx1 mouse model for preclinical research.

- **North American and Australian RUNX1 and Inherited Hematologic Malignancy Predisposition Consortium** launches.

- Patient community increases to 121 families and 230 patients.

- Research funding increases to $9M total to support 21 research groups and 31 projects.

- Two independent RRP-funded investigators describe the prevalence of germline *RUNX1* mutations in *RUNX1*-mutated AML as between 10-30%.

- A cross-institutional, cross-disciplinary working group forms to draft the first clinical trial protocol to test imatinib for *RUNX1*-FPD.

- **STRIVE Grant Program** launches with The Mark Foundation for Cancer Research.

- Director of Development and Communications Associate join the RRP team.
2020

- RUNX1 database reports 164 unique RUNX1 germline mutations, representing 259 families.
- EU Network on Familial Platelet Disorder with Cancer Predisposition launches.
- RRP launches educational webinars.
- Dr. Nancy Speck uncovers that neutrophils in a Runx1 deficient mouse model are important contributors to inflammation.
- Annual Patient Meeting launches as an integration with the RUNX1 Annual Scientific Conference.
- RRP and the Edward P. Evans Foundation establish the FIRE Grant Program.
- Research Guided by Patients Committee (RGPC) launches to focus on developing patient-centered outcomes RUNX1-FPD research.

2018 - 2019

- RRP launches a communication campaign in support of the NIH RUNX1 Longitudinal Clinical Research study led by Dr. Paul Liu.
- RRP and Alex’s Lemonade Stand Foundation launch collaborative Early Career Investigator Grant Program.
- RRP establishes the RUNX1 database with leaders Drs. Anna Brown and Hamish Scott.
- RRP connects with 12 families and 15 patients.
- Patient Engagement and Clinical Programs Manager joins the RRP team.
• RRP launches inaugural RUNX1 Scientific Research Conference.

• Alex’s Lemonade Stand Foundation and RRP announce cofunded annual grant program.

• The Leukemia & Lymphoma Society partners with RRP to create a cofunded research grant.

• The literature catalogues less than 70 germline RUNX1 mutations

**2016: Tim & Monica Babich co-founded RRP.**

**Social Media & Website Reach**

![Graph showing annual website visitors and social media followers](image)

Top 10 Website Visitor Countries:
WE ARE NIMBLE

Unlike other medical research organizations, RRP is able to pivot quickly. We create grant programs designed to fill critical gaps in RUNX1-FPD research, knowledge and expertise.

Aside from our Early Career Investigator program in collaboration with Alex’s Lemonade Stand Foundation (ALSF), we rarely administer the same grant program year after year.

That’s because we continuously evaluate the scientific landscape, identify the critical questions that need to be answered and design a program to fit the purpose.

This approach has the following advantages:

1. It expands our network of co-funding partners and multiplies dollars spent on RUNX1-FPD research;
2. It broadens our outreach to new scientific investigators whose research efforts could impact RUNX1-FPD;
3. It focuses our funding on specific areas of interest where the need for research dollars is greatest.

In 2021, in collaboration with ALSF and two new co-funding partners, the Edward P. Evans Foundation MDS Initiative and the Mark Foundation for Cancer Research, we approved funding for seven new research projects through three different grant programs totaling more than $1.9M in grant funding.

"RRP does things differently - applying dollars to fill the biggest knowledge gaps to guide us in the right direction on our road to cancer prevention for RUNX1-FPD."

~ Dr. Katrin Ericson, RRP Executive Director
"[There is] a real commitment to work together. The Foundation has done an amazing job to foster a community spirit and a desire to work towards a common goal."
~ Dr. Nancy Speck, Chair, Scientific Advisory Board

WE ARE COLLABORATIVE

Since our founding, RRP has fostered collaboration in our community, building a diverse Scientific Advisory Board with leaders in the fields of RUNX1 biology, clinical trials and biotech who guide our research portfolio.

Two patient-driven advisory bodies - the Patient & Family Advisory Council and the Research Guided by Patients Committee - bring forth the “patient voice” and help shape our education, advocacy and outreach initiatives, including educational webinar topics, patient resources, awareness building and fundraising initiatives.

We have also spearheaded the establishment of two consortia: the 36-member North American and Australian RUNX1 Inherited Hematologic Malignancies Consortium and the EU Network for Platelet Disorders with Predisposition to Hematologic Malignancies.

By creating multiple opportunities for our research community to share data and by promoting scientific exchange, we are able to accelerate science beyond just direct funding.

We have created sharing forums and established databases to help ensure that time and money are not wasted on duplicative efforts due to research silos, or experiments that are unnecessary given new knowledge gained from another research group.

Not only do we host an annual conference each year, but we offer research webinars and facilitate working group meetings to encourage dialogue among labs working within a similar research area in order to provide an opportunity to share results and offer feedback as often as possible.
To ensure the speeding up of understanding the number of RUNX1 mutations that cause disease and to identify the type of mutations that lead to cancer, we funded the creation of the world's largest RUNX1 database that catalogues RUNX1 mutations and somatic mutations from patients across the world.

WE ARE LASER FOCUSED

Focus is an essential principle for RRP. Our mission is always top of mind.

There are many interesting questions to address about any given disease or gene, especially a transcription factor like RUNX1 that itself can control many different genes within many diverse cell types.

However, we've maintained laser focus on funding research that will support the discovery of a cancer prevention, not a cancer therapeutic for patients with AML and RUNX1 mutations, nor a new treatment for eczema in RUNX1-FPD patients.

We are dedicated to funding ONLY studies that aim to stop the first cancer cell from taking hold.

How Studying RUNX1-FPD Could Help Advance Cancer Prevention Research
2021 FUNDED RESEARCH PROJECTS

Our 2021 research portfolio saw the inclusion of seven scientific research studies to discover novel cancer prevention treatments.

It has long been accepted in the scientific community that chronic inflammation combined with a dysfunctional immune system is associated with the development of cancer.

Ongoing studies of RUNX1-FPD patients have suggested that systemic inflammation and a dysfunctional immune system are in fact common in our disease.

Our 2021 funded research projects are designed to uncover if inflammation and an abnormal immune system contributes to cancer initiation and acceleration in RUNX1-FPD.

This knowledge could lead to the discovery of safe, early cancer prevention interventions for OUR patient community, and may have the added bonus of universally applying such discoveries for cancer prevention in other individuals.

The next page contains a table of RRP’s 2021 funded-research projects with links to summary descriptions for each.
<table>
<thead>
<tr>
<th>Grant Awarded</th>
<th>Grantee &amp; Organization</th>
<th>Project Title</th>
<th>Amount Awarded</th>
</tr>
</thead>
<tbody>
<tr>
<td>RRP-ALSF ECI Grant</td>
<td>Waihay Wong, MD, PhD</td>
<td>“Characterizing Inflammatory Phenotypes Associated with RUNX1 Deficiency”</td>
<td>$180,000</td>
</tr>
<tr>
<td></td>
<td>Brigham &amp; Women’s Hospital</td>
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<td>3 years</td>
</tr>
<tr>
<td>RRP-ALSF ECI Grant</td>
<td>Serine Avagyan, MD, PhD</td>
<td>“Role of PHF6 Mutations in Germine RUNX1 Deficiency Associated Hematopoietic</td>
<td>$180,000</td>
</tr>
<tr>
<td></td>
<td>Dana-Farber Cancer Institute/Boston Children’s Hospital</td>
<td>Disorders”</td>
<td>3 years</td>
</tr>
<tr>
<td>RRP-MDS FIRE Grant</td>
<td>Anupriya Agarwal, PhD</td>
<td>“Modeling RUNX1-FPD to Identify Interventions Suppressing Myeloid</td>
<td>$400,000</td>
</tr>
<tr>
<td></td>
<td>Knight Cancer Institute at Oregon Health &amp; Science</td>
<td>Expansion and Clonal Evolution”</td>
<td>2 years</td>
</tr>
<tr>
<td></td>
<td>University</td>
<td></td>
<td></td>
</tr>
<tr>
<td>RRP-MDS FIRE Grant</td>
<td>Alexander Bick, MD, PhD</td>
<td>“Identifying Anti-Inflammatory Therapeutics That Prevent Clonal Hematopoiesis</td>
<td>$400,000</td>
</tr>
<tr>
<td></td>
<td>Vanderbilt University Medical Center</td>
<td>Progression in Humans”</td>
<td>2 years</td>
</tr>
<tr>
<td>RRP-MFCR STRIVE Grant</td>
<td>Lucio Castilla, PhD</td>
<td>“Inflammation and Predisposition to Hematologic Malignancies”</td>
<td>$250,000</td>
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<tr>
<td></td>
<td>UMass Chan Medical School</td>
<td></td>
<td>18 months</td>
</tr>
<tr>
<td>RRP-MFCR STRIVE Grant</td>
<td>Lucy Godley, MD, PhD</td>
<td>“Delineating the Epigenetic Landscape and Pro-inflammatory Gene Expression</td>
<td>$250,000</td>
</tr>
<tr>
<td></td>
<td>The University of Chicago</td>
<td>Program Associated with Familial Platelet Disorder”</td>
<td>18 months</td>
</tr>
<tr>
<td>RRP-MFCR STRIVE Grant</td>
<td>Ravi Majeti, MD, PhD</td>
<td>“Characterizing and Targeting Inflammation in RUNX1-Mutant Cancer Progression”</td>
<td>$250,000</td>
</tr>
<tr>
<td></td>
<td>Stanford University</td>
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<td>18 months</td>
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**TOTAL AWARDED**

$1,910,000

* RRP-ALSF ECI Grant: Awards funds to young investigators whose projects support the discovery of interventions that would prevent high-risk RUNX1-FPD patients from developing blood cancers.
* RRP-MDS FIRE Grant: Supports projects that address whether modulating inflammatory processes using available anti-inflammatory drugs can halt disease progression from clonal hematopoiesis to MDS and AML.
* RRP-MFCR STRIVE Grant: Supports innovative studies aimed to delineate the role of inflammation, the immune system and clonal evolution from CHIP to malignancy in RUNX1-FPD.
MEASURING OUR RESEARCH IMPACT

- 1 Clinical Trial in Planning
- 31 Research Projects Funded
- 5 Working Groups & Collaborations Initiated
- 6 Webinars with 500+ Attendees
- 3 Advisory Bodies/Consortia
- 5 Annual Conferences
- 22 Researchers Engaged
- $9 Million+ in Research Funding

STIMULATING RESEARCH TRANSLATES TO MORE PUBLICATIONS AND NEW KNOWLEDGE GAINED

First Grants Funded By RRP

<table>
<thead>
<tr>
<th>Year</th>
<th>Grants Funded</th>
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<tbody>
<tr>
<td>2016</td>
<td>14</td>
</tr>
<tr>
<td>2017</td>
<td>14</td>
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<tr>
<td>2018</td>
<td>11</td>
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<td>2019</td>
<td>23</td>
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<td>2020</td>
<td>25</td>
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<tr>
<td>2021</td>
<td>23</td>
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*PubMed Search Results For “Germline RUNXI”*
2021 X-OUT CANCER CHALLENGE CAMPAIGN

In February 2021, the RUNX1 Research Program launched the X-Out Cancer Challenge Campaign on its fifth anniversary.

The Campaign aimed to increase awareness of inherited blood cancers, draw attention to RRP’s cancer prevention focus and to encourage participants to help meet their health goals while helping to raise funds for RRP’s research and patient-focused initiatives.

Since many cancers have an environmental component, and it is well-established that moving more, eating healthier foods and living lower-stress lives contribute to better health outcomes, participants who joined the Challenge pledged to “X-Out” a bad habit (i.e., sugar, packaged foods, social media, alcohol, inactivity) for the entire month, and then shared their Challenge page online.

The Campaign tackled both issues by affording individuals a way to do something positive for themselves while contributing to vital cancer prevention research.

Thanks to the generosity and support of our community, we raised $25,000 during the Campaign!
2021 RUNX1 WEEK ANNUAL SCIENTIFIC CONFERENCE AND PATIENT MEETING

Our 2021 annual scientific conference was held virtually for the second year in a row with a theme of Creating Connections, which is something we felt we’d all come to value as we supported each other during the global pandemic.

Our online conference engaged scientists from around the world, with more than 150 scientists, clinicians and researchers from ten different countries joining the 22 scientific session talks focused on RUNX1-related topics and current research findings.

2021 was also the fifth anniversary of the RUNX1 Research Program, and we celebrated with a virtual Gala event featuring a musical performance, a talent show and an awards ceremony for clinicians, researchers and patients. We also held a silent auction, which raised more than $5,000 for cancer prevention research and patient-oriented programs.

Dr. David Fajgenbaum was our keynote speaker, and shared his incredible personal story of saving his own life from the rare Castleman’s disease through a deliberate drug repurposing strategy that aimed to identify FDA-approved drugs with relevant mechanisms of action as quickly as possible.

The second annual Patient Meeting was held over the weekend and included discussions on key takeaways from the scientific sessions, legislative advocacy and health & wellness resources.

We had over 25 patients and their family members join throughout the week to share their lived expertise and to learn about how they are integral to moving research forward.

“Thank you for introducing me to so many others who have made me realize that I am part of a larger community than we ever realized, [and] thank you for creating an integrated team of clinicians, researchers and patients, working together to advance knowledge of a disorder that was only identified in recent times.”

~ RUNX1-FPD Patient and Father of Patients
The RRP Scientific Advisory Board includes both academic and industry experts who span basic science, translational science, clinical research and clinical development.

Members offer advice and guidance on how best to propel clinical research forward with the goal of positively impacting our patients’ lives as quickly as possible, and play a critical role in defining our research agenda, reviewing grant applications and fostering collaborative relationships within the scientific community.
The RGPC advises RUNX1 Research Program’s activities, with a focus on the research arm of our mission. This committee includes patients and caregivers, along with clinicians and researchers, dedicated to developing RUNX1-FPD research centered around patients’ defined priorities and preferred outcomes.

For the first two years of this effort (2020-2021), the RGPC was funded by a Patient Centered Outcomes Research Institute (PCORI) Eugene Washington Engagement Award to develop a training program that will support any RUNX1-FPD patient or caregiver to effectively participate as a partner in this research endeavor.

The PFAC includes a group of patients and caregivers who have committed to sharing their diverse experiences and perspectives to advise on the RUNX1 Research Program's activities.

RRP staff look to them to represent the “patient voice” to prioritize education, advocacy and outreach initiatives, including educational webinar topics, patient resources, awareness building and fundraising initiatives.

The North American & Australian RUNX1 and Inherited Hematologic Malignancies Consortium brings together physicians and genetic counselors who care for RUNX1-FPD and similar inherited platelet disorder patients across the U.S. and Canada in order to drive collaboration and accelerate advancements in medical care for patients. This list of experts is available to both patients and medical professionals for consultation. Visit our website for direct contact information.
PARTNERS & CO-FUNDERS

We are fortunate to have these organizations as partners and co-funders. Thank you for your support!

Alex’s Lemonade Stand
Evans MDS
Leukemia & Lymphoma Society
The Mark Foundation for Cancer Research

Thank you to our webinar promotion partner!

mds foundation

Ways To Support RRP

To make a donation or learn about ways to help support our mission, go to our Donate webpage: www.runx1-fpd.org/donate.

Or contact Alex Gonzalez, Director of Development
agonzalez@runx1-fpd.org
FINANCIALS

2021 Support & Revenues

- Donor Advised Funds: $2,847,000
- Contributions: $2,051,778
- Grants: $350,000
- Government (PPP): $50,695
- Events & Campaigns: $37,269

Total Revenues: $5,336,742

2021 Expenses

- Research & Support Grants: $1,500,912
- Program Expenses: $299,996
- Total Program & Research: $2,330,908
- Management & General: $140,561
- Fundraising: $116,797

Total Expenses: $2,588,266

Ending Net Assets

$2,747,823

as of 12/31/2021