

The Importance of Tissue Donation

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DTRF Annual Patient Meeting - September 23, 2017

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Why is RCRF partnering with DTRF?

The Rare Cancer Research Foundation is a non-profit dedicated to curing rare cancers by creating shared resources for the cancer research community. It was founded by a rare cancer patient who wanted to speed the research process for ALL rare cancers, not just his own.



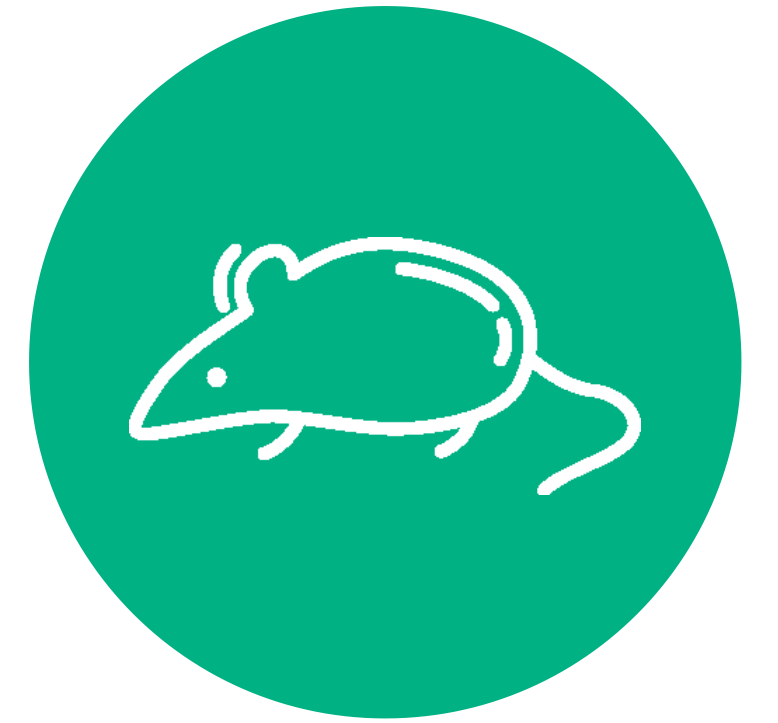
Although Desmoid Tumors aren't considered 'cancer'
they have many **similarities** to malignancies
they are **rare**
they utilize similar **treatments**
they can cause significant life **disruption** & even death
they have similar **research** needs

The Importance of Tissue Donation

“Donation of tissue by individuals and patients is an immensely important gift to the research process. Medical researchers use tissue donations to make important discoveries that improve the detection, diagnosis, and treatment of cancer.” - <https://cancergenome.nih.gov>

Disease models, created from patient tissues, enable scientists to

- Better understand underlying **biology** of the disease
- Test & identify ways of **diagnosis & prevention**
- Test & identify new **therapies & potential cures**



The Importance of Tissue Donation

Key Issues in advancing research for rare cancers/diseases include

- **Limited patients** presented at single institution
- **Stored tissue** may not be fully utilized
- Lack of **research access** to tumor tissue
- Lack of robust **model systems**
- Lack of **open source** models & data



Patients can become “partners in research” through tissue donation to help solve these issues!

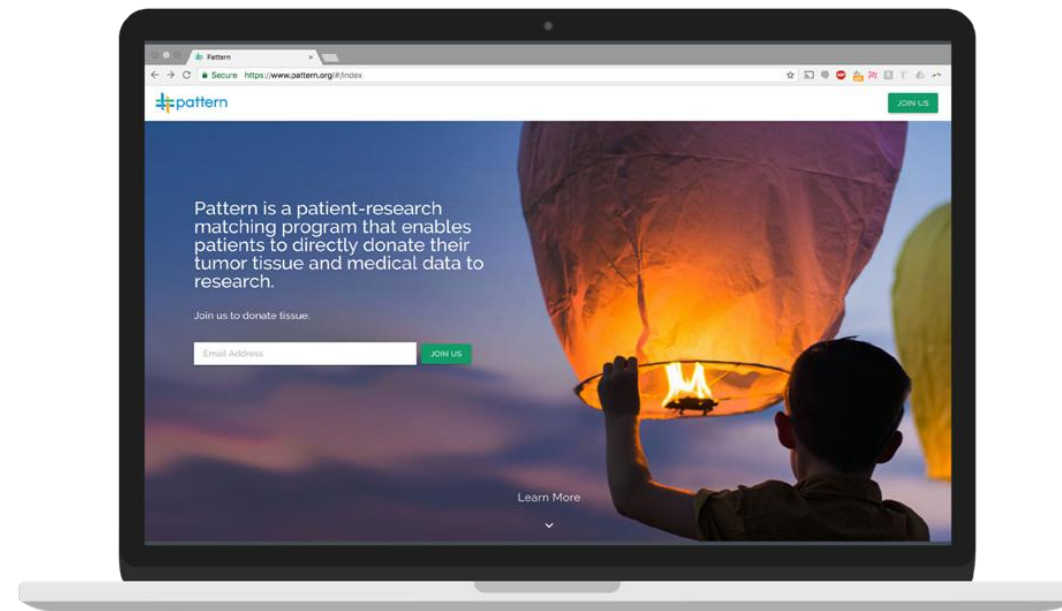


The Rare Cancer Research Foundation launched pattern.org **to enable** patients to **directly** donate their tumor tissue and medical data to high impact research. Pattern.org **bridges the gap** between patients and scientists, **empowering** patients to be partners in research.



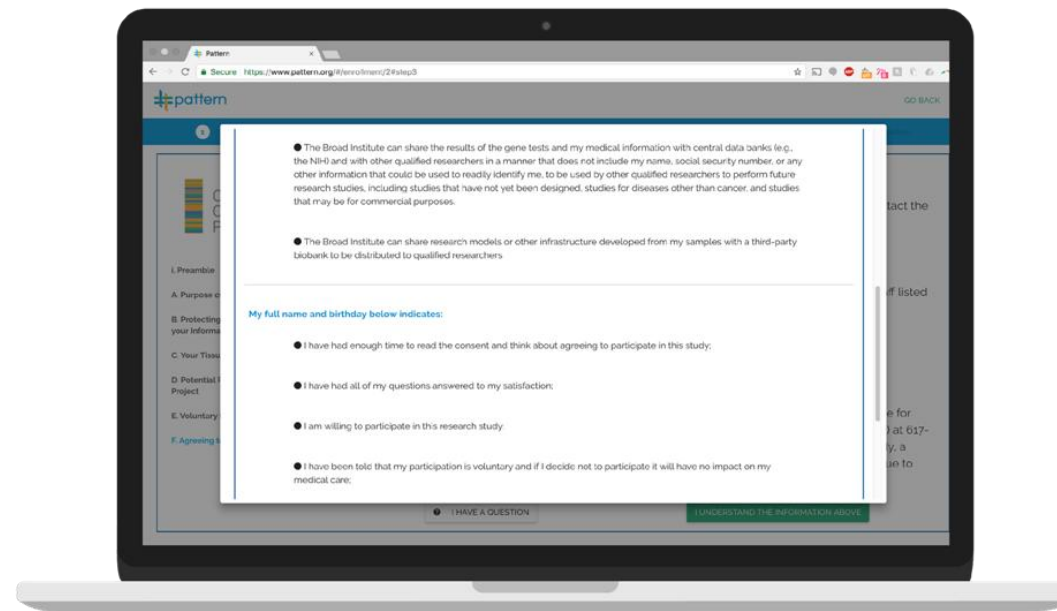
How pattern.org works

1



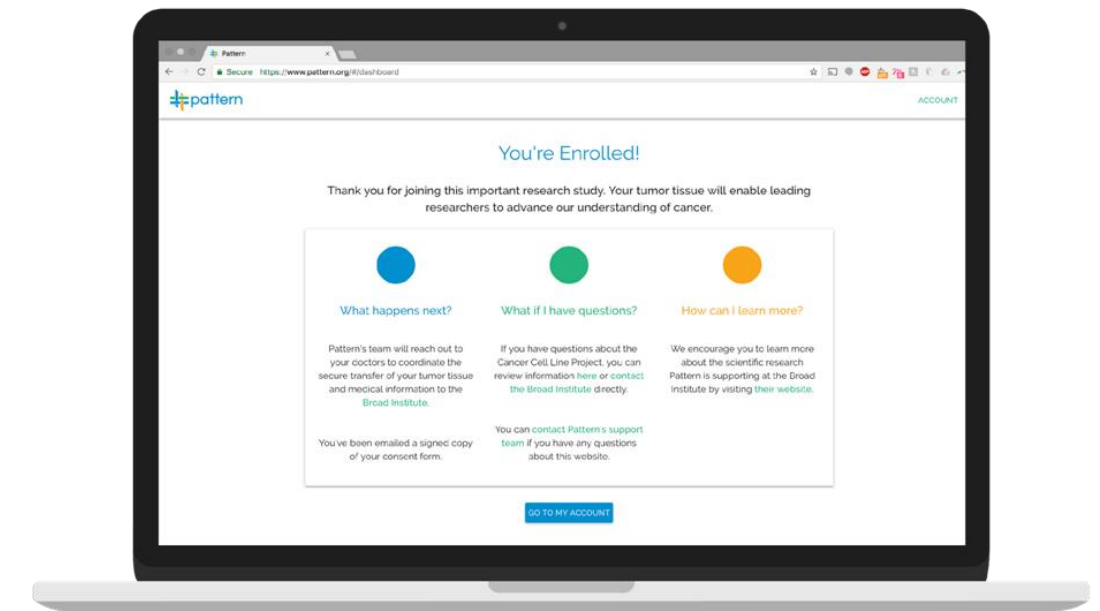
Patient registers with basic info then learns about an applicable research study

2



Patient with upcoming surgery or biopsy consents online

3



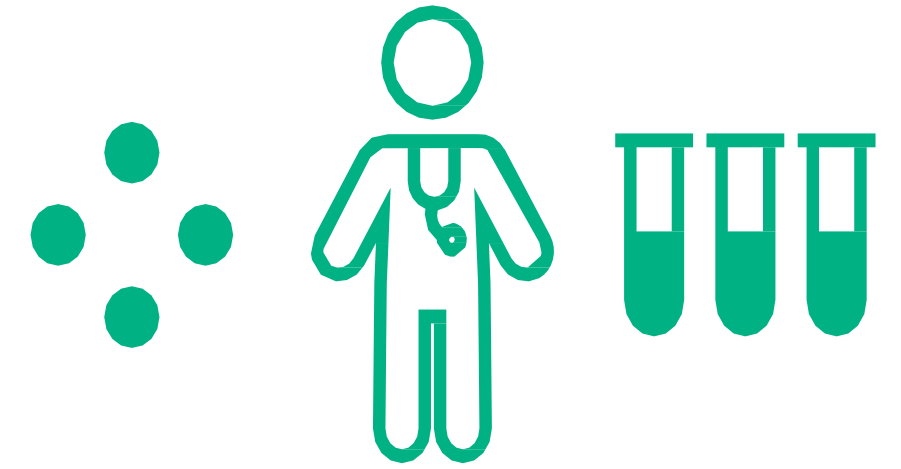
pattern.org does the rest by coordinating tissue and data transfer

Pilot Collaboration with the Broad

Desmoid patients, along with 10 cancer indications, are using pattern.org to direct their tumor tissue to the **Cancer Cell Line Project (CCLP)** at the **Broad Institute of MIT and Harvard**, a world-renowned research institution. The fresh tissue and patient data is **de-identified**, then sent to the Broad where it will be used to **develop models** (cell lines or organoids). Models will be **validated & genetically sequenced** then placed into a **non-profit biorepository (ATCC)** to be **shared openly** with the worldwide research community.



The Importance of the Broad/Pattern.org Pilot



- What are Cell Lines and why are they important?
 - Cell lines are a group of cells derived from one cell or set of cells of the same type and which under certain conditions the cells proliferate virtually indefinitely in the laboratory
 - They are the most widely used models to study the biology of cancer and to test therapeutic strategies
- What's an Organoid?
 - A tumor organoid is a 3-dimensional structure derived from tumor samples that are maintained and grown in a laboratory
 - They also are used as models to study the biology of cancer and to test therapeutic strategies



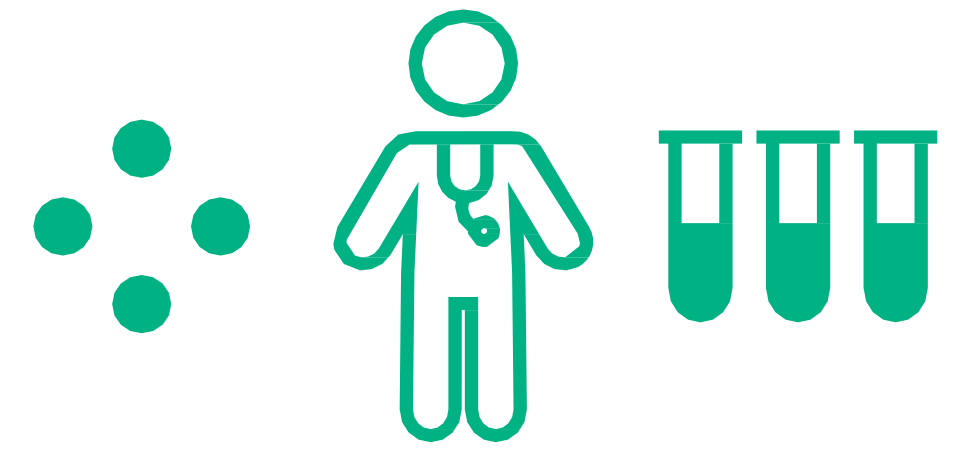
The Importance of the Broad/Pattern.org Pilot

Helps address key issues in advancing research for Desmoid Tumors



- **Limited patients** presented at single institution
 - Tissue can be collected at **any continental U.S. institution**
 - Tissue which may have been tossed is **utilized for research**
- **Stored tissue** may not be fully utilized
 - Donated **Fresh tissue** will be utilized
- Lack of **research access** to tumor tissue
 - Pattern.org delivers to **Broad researchers**
- Lack of robust **model systems**
 - The Broad creates models (**cell lines & organoids**)
- Lack of **open source** models & data
 - Resulting **models & data placed in public domain** to be shared **with other researchers**

Key Points of Broad/Pattern.org Pilot



- Utilizes **fresh tissue** from biopsy or surgical resection
 - **Excess** tissue only
 - **Won't affect clinical care**
 - Sample size 0.5 cm³
- Must consent **PRIOR** to surgery
 - Consent at least one week in advance for best chance of collection
- For **General Research** only - no results returned to patient for clinical care
- **Patient Privacy is protected**
 - Patient Tissue & Data is **de-identified**
 - Data stored on **encrypted, HIPAA compliant** data server with restricted access
- **Can revoke consent** at any time
- **No cost** to patient

Broad/Pattern.org Pilot Desmoid Tumor results

Program to Date		May Collect	Not Collected					
Consented	Delivered	Pending Upcoming Surgery	Consented Post-Surgery	International (Non-US Institution)	Surgery Cancelled	Insufficient Tissue Available	Patient Withdrew Consent	Institution Refused to Release
11	4	2	2	1	1		1	

- **Desmoid patient results to date:**
 - 11 consents
 - 4 samples delivered
 - 2 more surgeries w/ planned donations next week
 - Pilot limited to 10 delivered samples

- **Most engaged & supportive community to date**
 - THANK YOU DTRF & Desmoid patient community!

- **Pattern.org has potential for future projects**



Q & A

Thank you DTRF &
past and future tissue donors!

