

Fibrolamellar Hepatocellular Carcinoma

Adolescent/Young Adult Liver Cancer

Presentation for SSA Compassionate Allowance



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FACES OF FIBROLAMELLAR

Your support brings a cure closer to those facing this rare and devastating teen/young adult liver cancer



Fibrolamellar Cancer Foundation



Fibrolamellar Hepatocellular Carcinoma

Aggressive Form of Liver Cancer: One of the deadliest

Ultra Rare Cancer : 1 in 5 million

Adolescent /Young Adult: 12- 30 year old typically

Survivorship rate : Less than 30 % (5 year)

Year of Life Lost: Approximately 40

No known cause: Victims are typically young with uncompromised livers

Most commonly referred as Fibrolamellar, FLC or FL-HCC



Faces of Fibrolamellar: Robert

18 years old, college freshman, just starting to work part time to cover his college costs

Flu symptoms for 2 months led to distended belly – finally diagnosed as Stage 4 FLC

Fast progression to heart and lungs, with emergency liver transplant; 28 days in hospital

Metastasis required chemotherapy which took a major toll on his young body

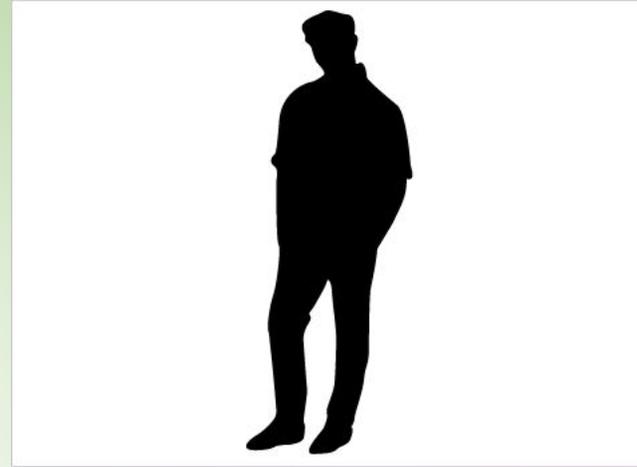
Unable to continue school, could not drive, quit the job he loved with not enough time to qualify for SSA benefits

Father quit his job to care for Robert- hoping to find a cure

Cancer progressed for 3 ½ years- with increasing disabilities, seven surgeries, numerous chemo regimens

Family exhausted their major savings to “save” Robert.

Sadly, we lost Robert in March 2017





Fibrolamellar Cancer Foundation

Founded in 2009

26 year old Tucker Davis of Greenwich, CT :

18 month battle

Died on Rare Disease Day 2010 at age 28





Mission



- Find a cure and treatment options
- Raise awareness of this disease
- Connect and support the fibrolamellar community of patients and their families

Research :2017 Fibrolamellar Summit

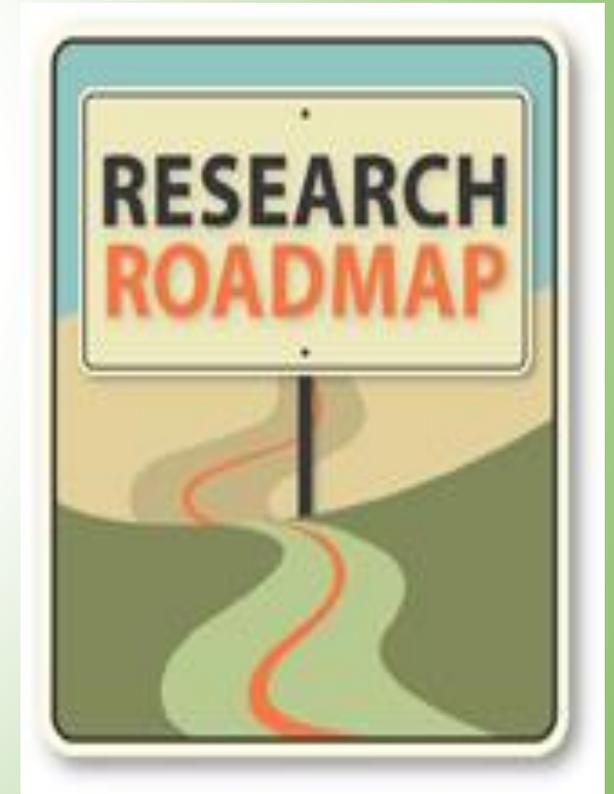
November 1st/2nd

Research Collaboration

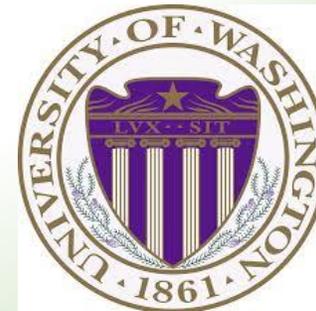
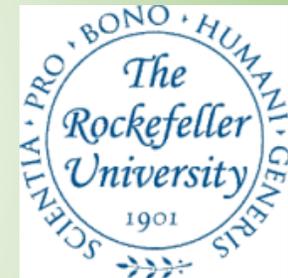
Participants from FL-HCC research community

- Academia
- Private Sector
- Government
- FCF Board
- Partner Foundations

\$ 7 million invested in research by the FCF



Research Partners



Fibrolamellar: Description

Rare, malignant tumor that arises from the liver

First described in 1956 and accounts for only 1% to 5% of all cases of classic hepatocellular carcinoma (HCC)

The tumor is typically characterized by fibrous bands in a unique “lamellar” pattern under the microscope

It is primarily diagnosed in patients from their young teens to late twenties.

Fibrolamellar: Description

What is the difference between HCC (hepatocellular carcinoma) and FLC (fibrolamellar)?

Like fibrolamellar, HCC is a malignant tumor of the liver. The similarities, however, stop there.

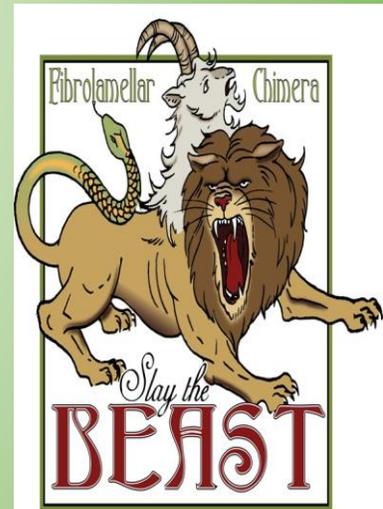
HCC - often associated with chronic inflammation of the liver, either by alcohol abuse or infection by hepatitis B or C.

Fibrolamellar - often occurs in the absence of alcohol or hepatitis infection, with hardly any sign of an inflammatory reaction in the liver.

-In fact, fibrolamellar patients tend to be younger, while HCC patients are older.

What causes fibrolamellar?

Recent research has shown a distinct mutation, common to all patients plays a key role in the formation of fibrolamellar. Fusion DNAJB1-PRKACA



Fibrolamellar: Diagnostic Testing and Coding

Difficult to diagnosis, primarily because its symptoms are attributed to more common causes.

Many patients complain of abdominal pain and fatigue, which can be caused by a multitude of things in the pediatric and adolescent population including the flu and a gastrointestinal virus.

Unlike HCC, there is no blood test available to raise a doctor's suspicion that the symptoms may be caused by something other than the flu.

For most patients with fibrolamellar, the diagnosis is made after an imaging study such as a CT-scan or MRI of the abdomen is performed, long after all other tests are completed and other causes ruled out.

Imaging studies will often reveal a large liver mass.



Treatments and Severity

Treatments: Surgery to remove the tumor and the surrounding lymph nodes is the standard treatment for fibrolamellar. Lymph nodes are removed as metastases (the spread of the cancer) to the lymph nodes is common.

There are no well-studied chemotherapy or other surgical alternatives.

Severity:

- Due to its rare nature, there are no large studies of fibrolamellar.
- Prognosis is often poor, particularly due to its late stage diagnosis
- Reported survival rates can range anywhere from 7 to 40 percent at five years.
- Complete surgical removal of the tumor improves this survival rate and also decrease the rate of recurrence.
- Patients whose tumors are not removed had an average survival of 12 months, while those who had tumors completely removed survived for an average of 112 months(4).

Faces of Fibrolamellar: Kristie

39 years old single mother of two young girls

Diagnosed with Stage 4 FLC over four years ago

Undergone three surgeries and numerous procedures and chemo regimens

Unable to work due to the advanced cancer stage and forced to quit her job to parent her daughters

Travels 50 miles on bus at 6 a.m. for chemo treatments, with her children by her side --- she had to sell her car

Due to lack of financial aid, set up "fund me" pages which only yield minimal donations to survive 1-2 weeks

Currently Kristie is in hospice, with little energy



Faces of Fibrolamellar: Ray

9 year old boy with unexplained bouts of nausea and vomiting

Misdiagnosed as acid reflux

Biopsy/scans revealed a large liver tumor with metastatic disease

Underwent a resection removing 60 % of his liver

While poor prognosis, at this age, the cancer, and all it's treatments can take years

Single mother is left to struggle between real need for income and Ray 's care and comfort, including ongoing treatments and medical visits

Child too young to contribute to SSA

Mother is faced with the hardship of how to finance their lives while providing all her time to saving Noah



Fibrolamellar Disability Criteria

13.19 Liver or Gallbladder

-- Cancer of the liver, gallbladder or bile ducts.

Key point: Liver cancer is already recognized by SSA Compassionate Allowance

Recommendations to SSA

- **List Fibrolamellar under 13.19**

Simply to add or specify this ultra rare form of liver cancer -fibrolamellar- to those 13.19 cancers currently listed

- **Work with our research and patient community** to know more about this ultra rare cancer
- **Help our patients and family** address the enormous financial and emotional burden this aggressive cancer poses

We Need Your Help: Take Action with Us!



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Thank you!

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Fibrolamellar Compassionate Allowance

Attachments

- ASCO Post 9/25/15 : FCF Spotlight
- Daily Mail, 5/13/15 Bronte's Story
- Rare Disease Report, 10/17/17: Fibrolamellar takes the lead on liver cancer awareness month
- Medscape, 9/17/15: Fibrolamellar overview
- Oncology, 9/19/13: Prognostic indicators and treatment outcome of FL-HCC
- Modern Pathology, 9/1/17: Molecular testing for the clinical diagnosis of fibrolamellar
- Gastrointestinal Research, Jan/Feb 2013: Clinicopathologic characteristics and survival outcomes of patients with fibrolamellar carcinoma
- Gastroenterology, 9/8/17: CRISPR/Cas9 Engineering of Adult Mouse Liver Demonstrates That the Dnajb1-Prkaca Gene Fusion is Sufficient to Induce Tumors Resembling Fibrolamellar Hepatocellular Carcinoma.