

Desmoid tumors

A focus on French Research Projects

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On behalf of the SOS-Desmoide Association

French research on DT is

- Developed under the auspices of the GSF group (French Sarcoma Group)
- Developed under the auspices of SOS DESMOIDE that provides non-financial and financial support to develop research and knowledge about DT
- Also supported by the French InfoSarcomes patients' advocacy group under a global coordination by InterSarc

Non-financial Support from SOS Desmoide

- Information of patients and caregivers
- Lobbying for DT research towards medical community
- Constant dialog with sarcoma experts from the French Sarcoma Group and InterSarc
- Fundraising from big companies

Different research areas

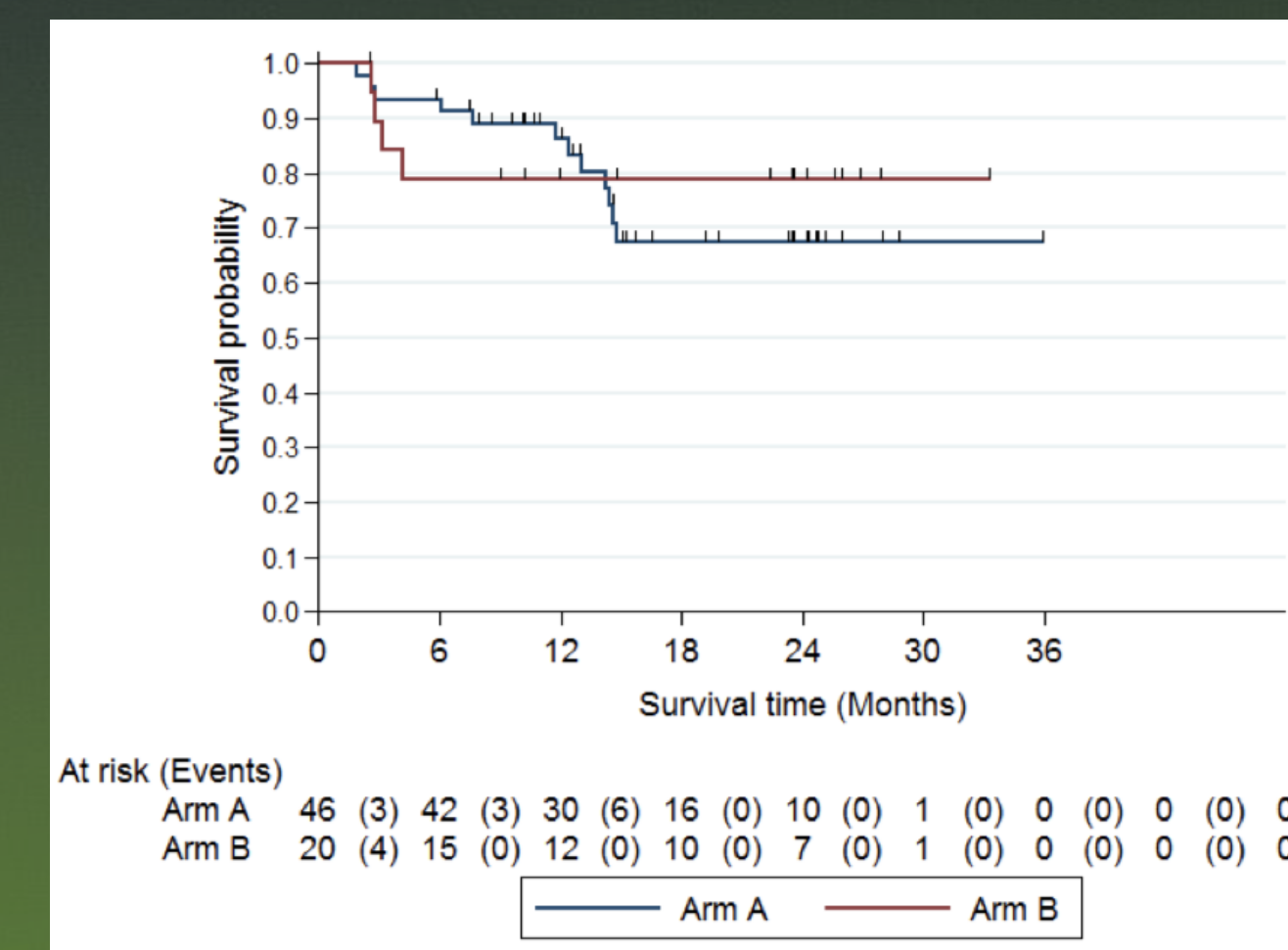
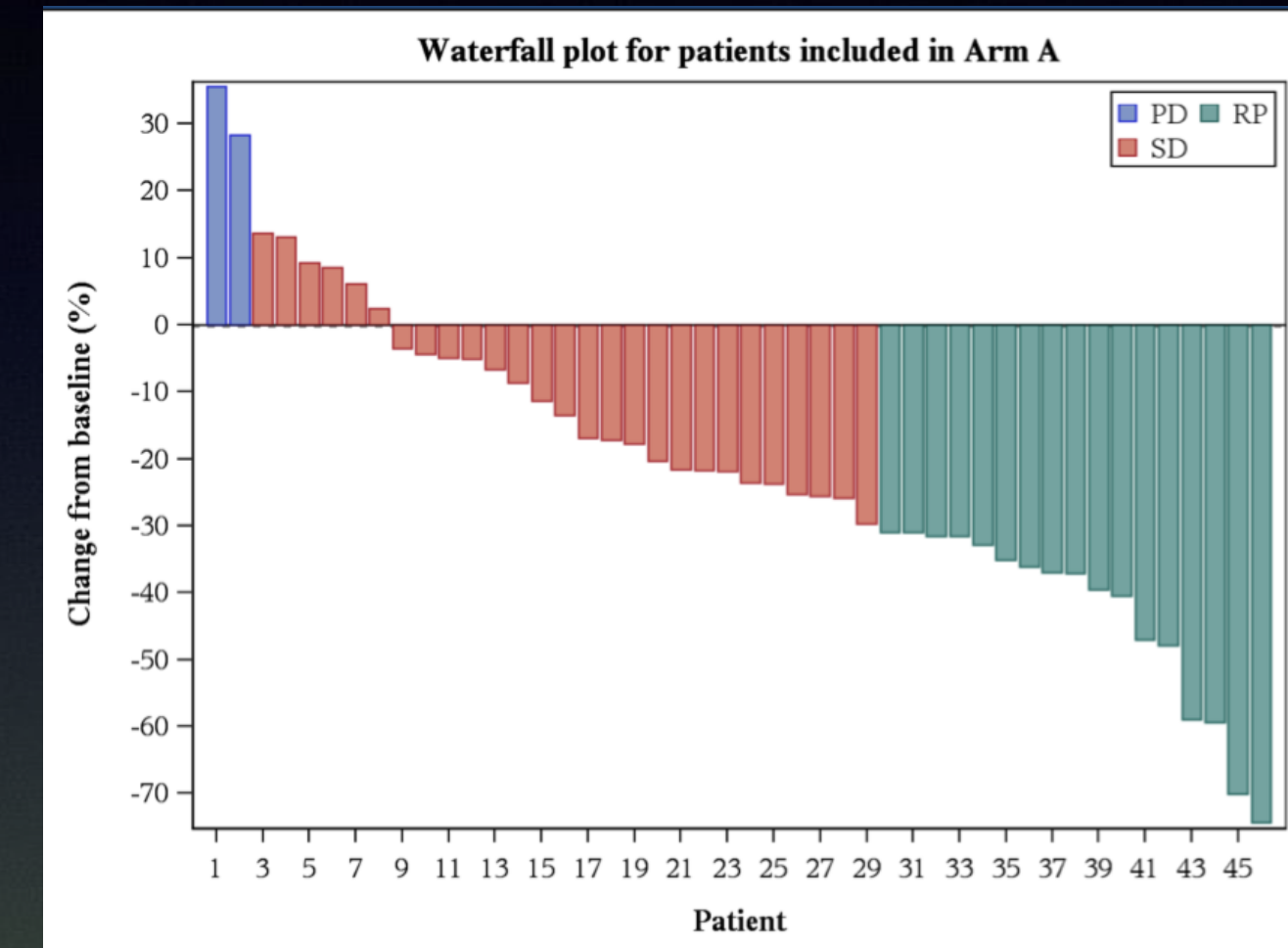
- From the bench to the bedside
- Basic science research (molecular signatures, animal models)
- Clinical research to investigate anti tumor compounds
- Non-pharmaceutical approaches to DT : the CRYODESMO projects
- Psycho-social research (QoL)

Past and running basic science research

- Gene expression profiling and correlation with PFS (Salas et al 2015)
- Identification of a blood biomarker for DT diagnosis (S Salas - 2015)
- Investigating the value of free circulating tumor DNA (Macagno et al 2018)
- Establishment of a murine DT model (F Chibon - 2019)

Clinical research: investigational compounds

- The DESMOPAZ trial (Pr A Italiano): investigating pazopanib versus MTX/VLB chemotherapy
- Presented at ASCO 2018 (Toulmonde M et al.)
- More than the results: randomized phase II trials proven feasible in France even for rare tumors



Clinical research: past and ongoing projects (non-pharmaceutical)

- The ALTITUDES cohort (Pr N PENEL): A prospective cohort aiming to enroll 300 patients to decipher clinical, molecular and epidemiological data on DT, linked to tumor sampling
- The wait and see study funded by the French National Cancer Institute (S Bonvalot)
- Psycho-social research on self-representation of the disease and patients' experience (C Flahault)
- Quality of life and functional consequences in DT children (G Duhil de Benaze)


Cryoablation for Desmoid tumors

- Focal therapy for benign & malignant tumors
- Promising in DT since the first report by the Mayo Clinic team
- Does not have the pitfalls of surgery for DT and has advantages over radio frequency techniques towards local inflammation
- Developed in several French centers for localized kidney cancers

Basics of cryoablation

- Thomson-Joule Effect
- 300 Bar argon
- Helium to warm
- Small probes
- Compatibles CT/MRI

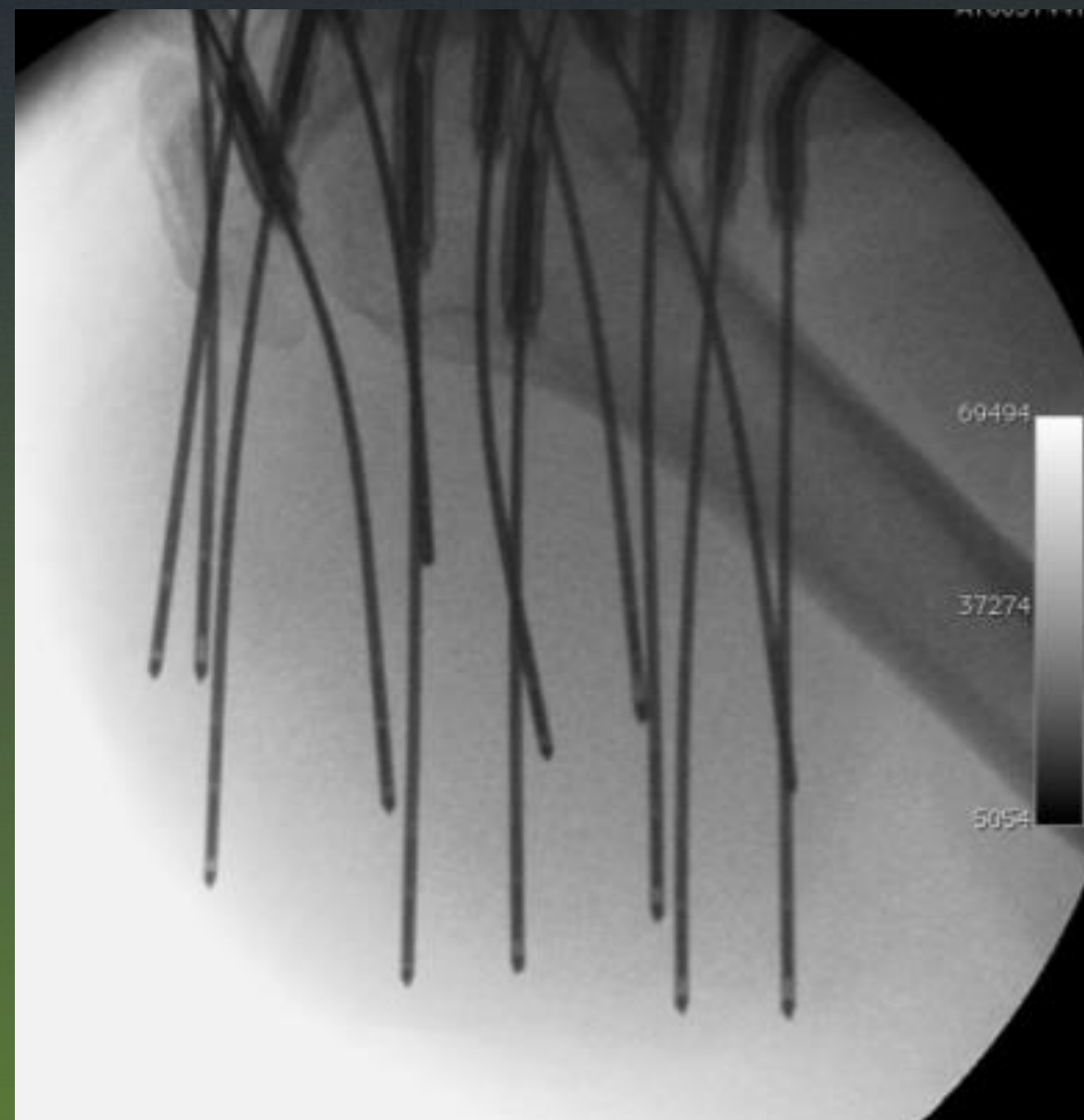
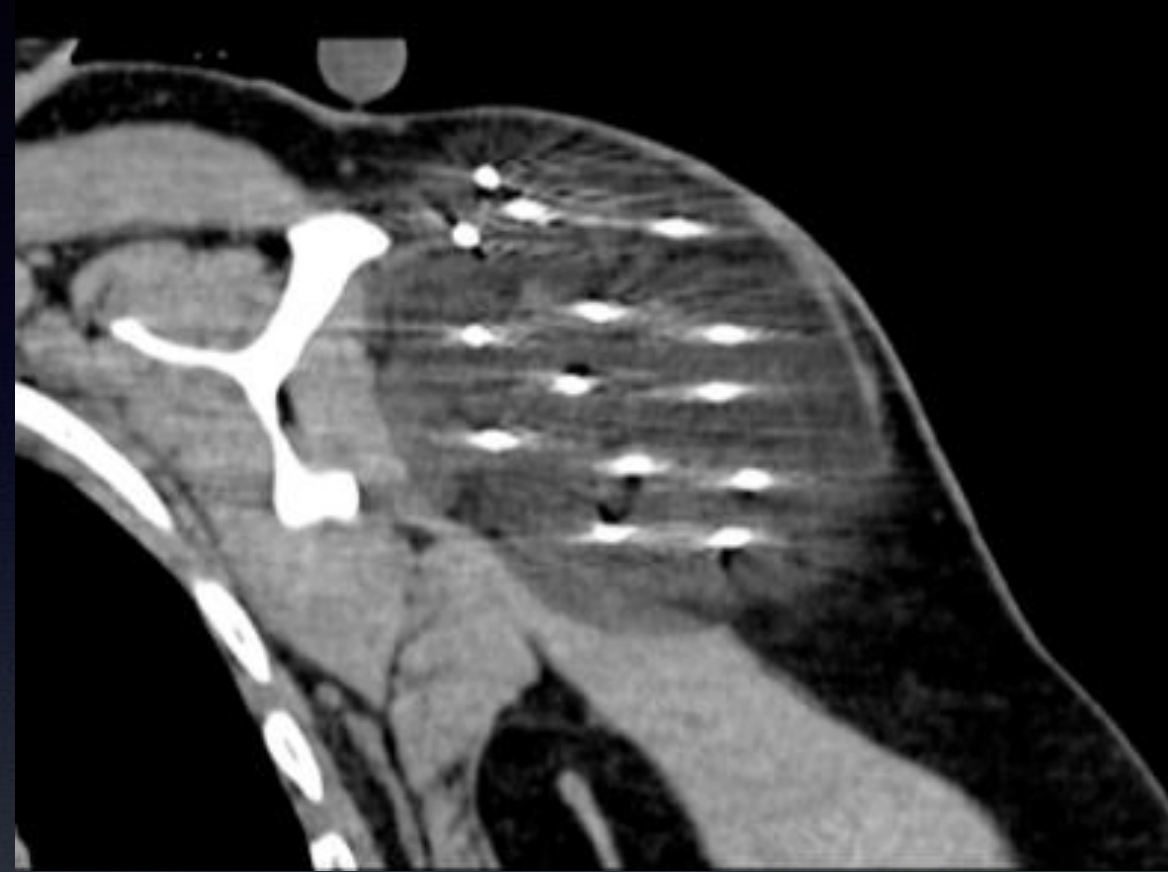
Song of Cold Genius
From the Opera "King Arthur"
H Purcell



Arranged for a 3 Octave Set of Hand Bells & Soprano Voice



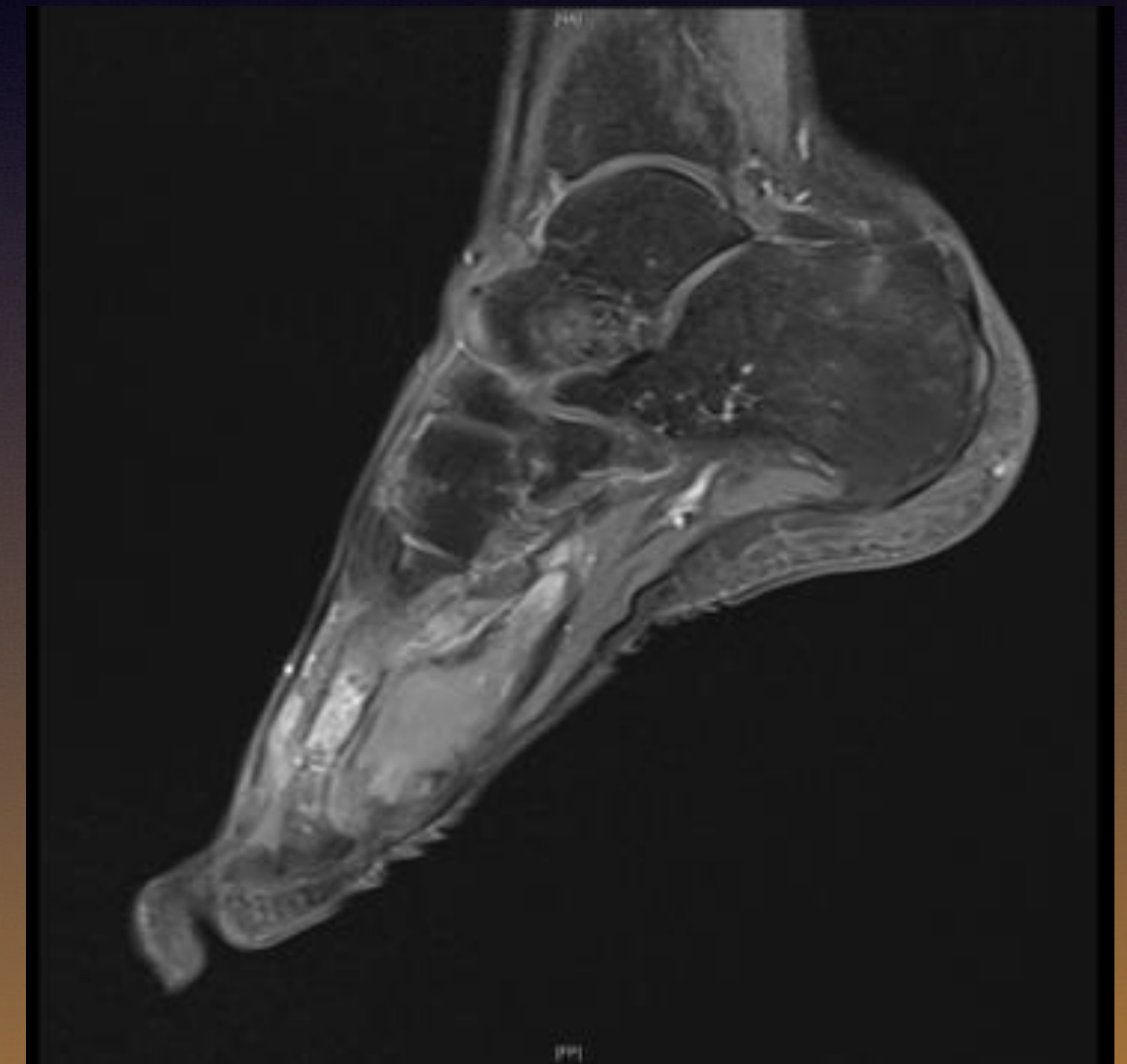
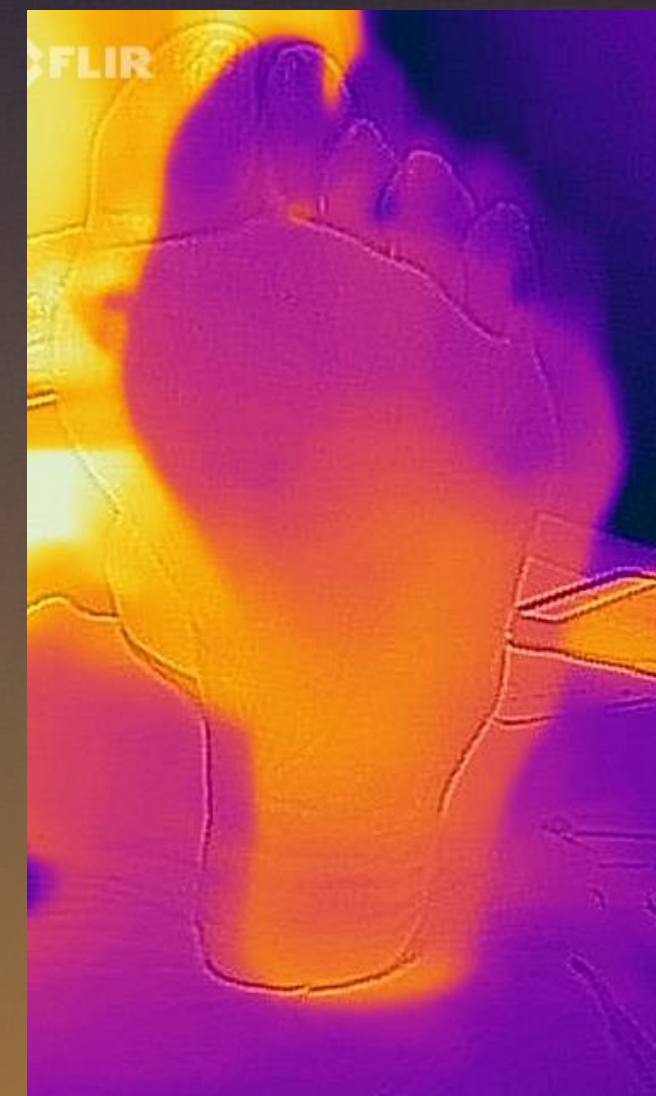
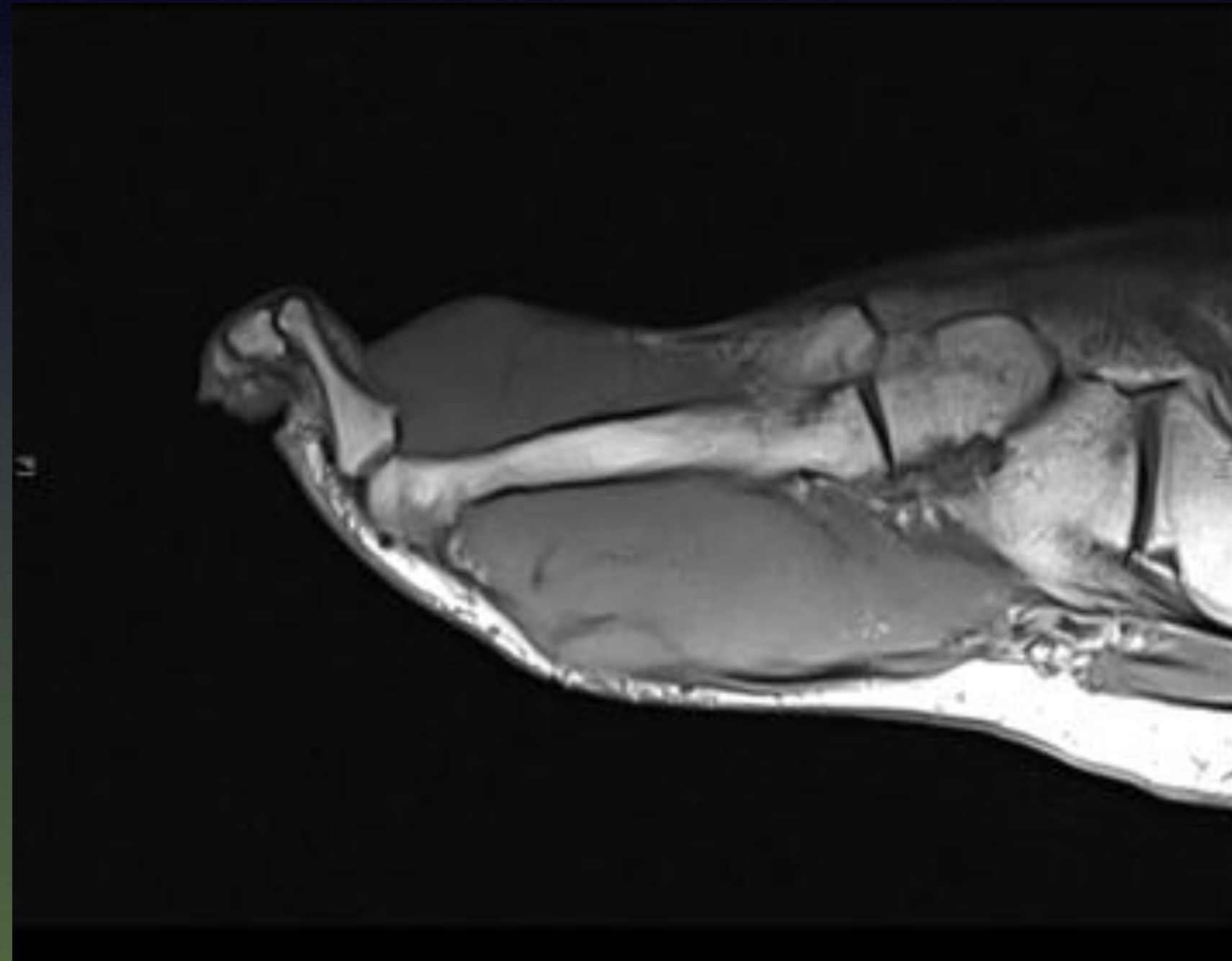
Into the procedure



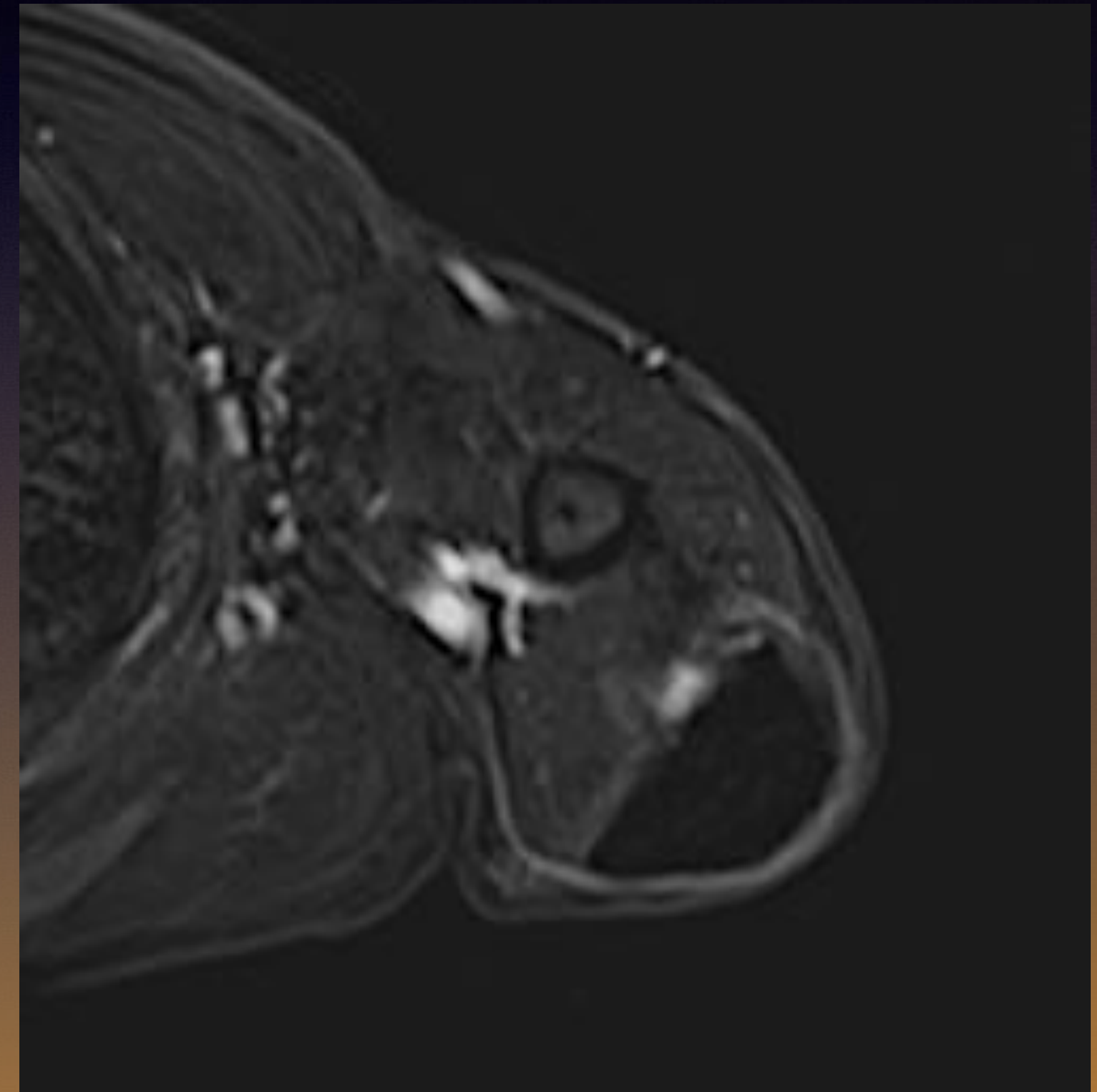
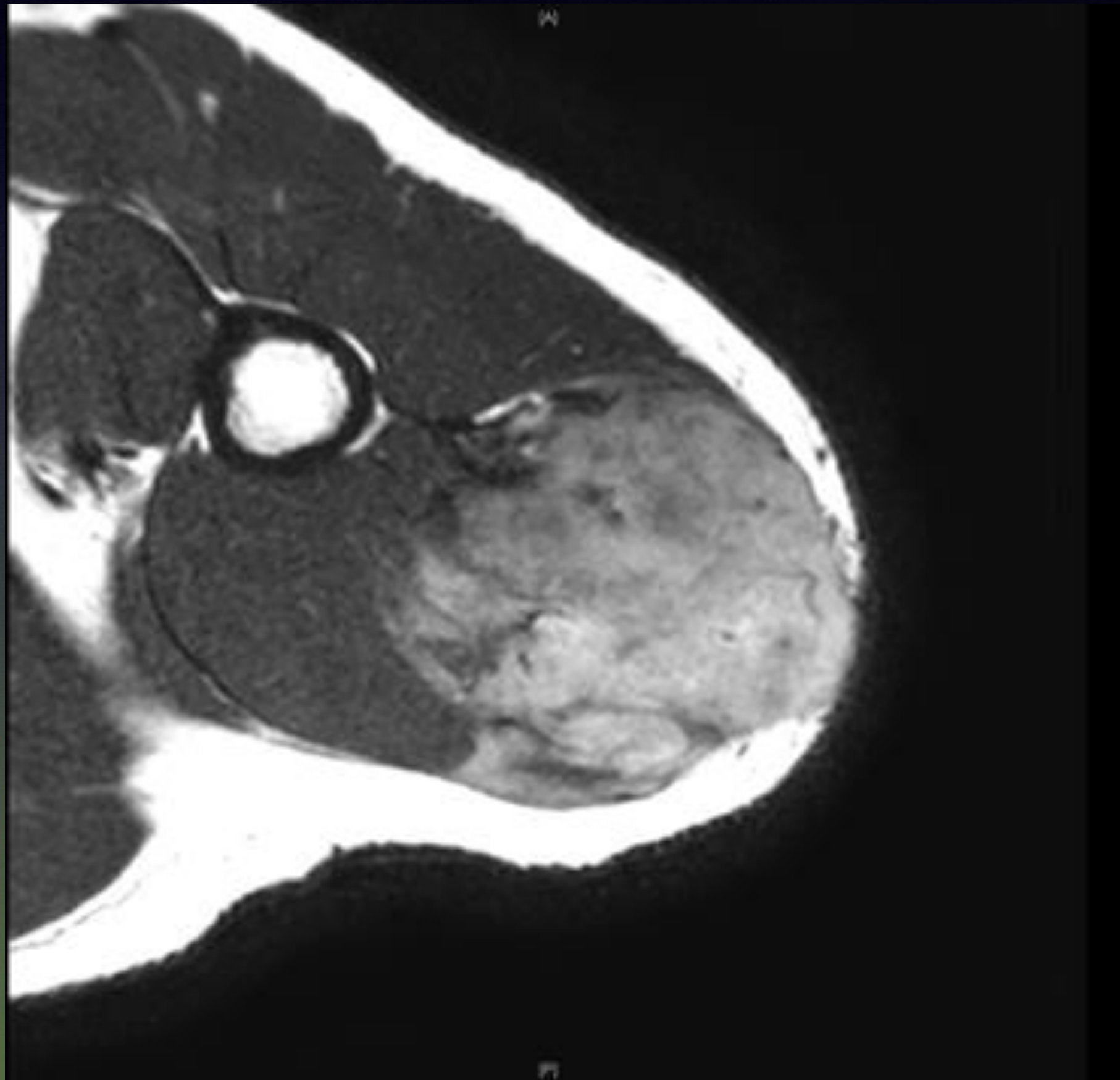
OMG: It's cold !



Cryoablation **examples**



Cryoablation **examples**



The CRYODESMO program

- **CRYODESMO-01** was a phase II study funded by the French National Cancer Institute, lead by the Strasbourg University Hospital team (Prs Gangi & Kurtz)
- 50 patients with progressive DT not responding to at least 2 prior medical treatments were enrolled
- Results are pending (database lock scheduled for Q1, 2019)
- Very promising responses w/ tumor shrinkage and symptom improvements

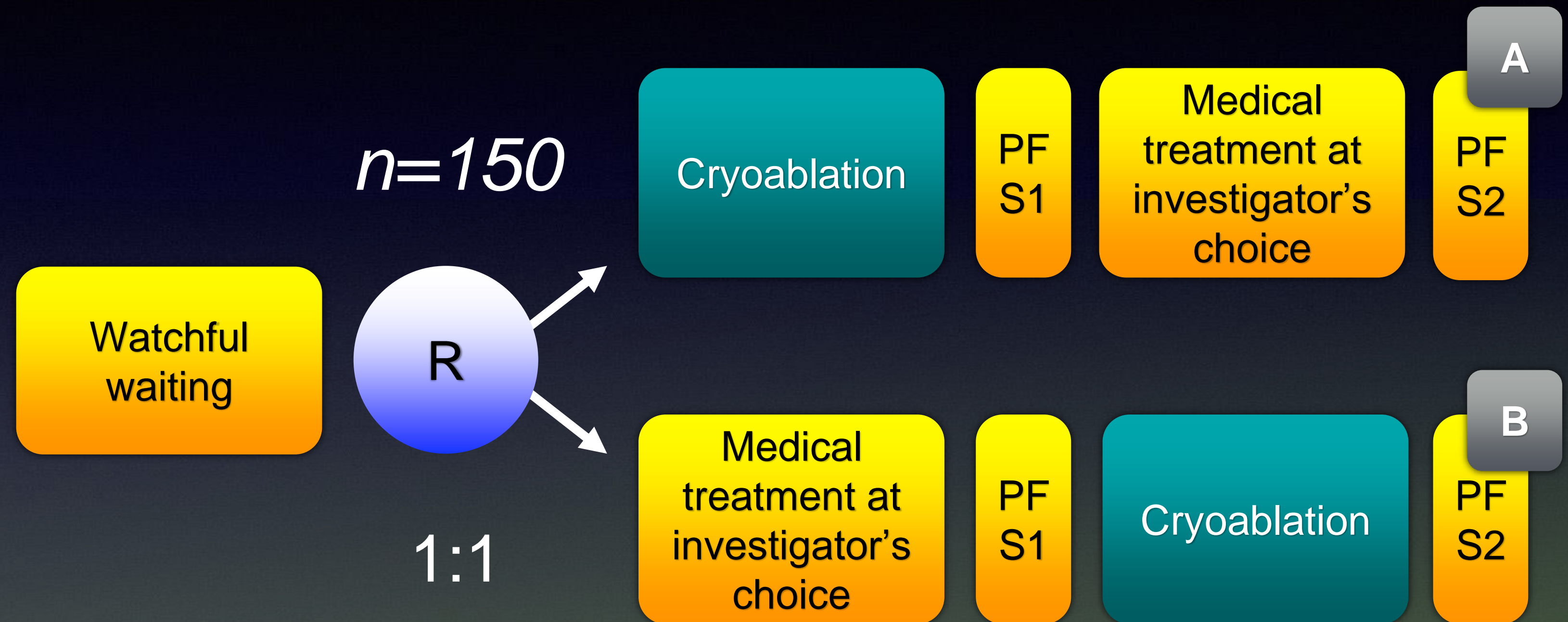
CRYODESMO-02 is coming

- **CRYODESMO-02** is a randomized phase II study submitted to the French National Cancer Institute funding program, lead by the Strasbourg University Hospital team (Prs Gangi & Kurtz)
- **CRYODESMO-02** aims to put forward cryoablation to less advanced, since symptomatic DTS

CRYODESMO-02 is coming

- Pathologically proven DT
- Extra-abdominal
- Progressing or symptomatic after WW
- Deemed accessible to complete cryoablation
- Adults & adolescents >12

Any prior medical therapy or surgery



Primary endpoint

- Superior PFS 1 (A vs B)

Secondary endpoints

- Safety, QoL
- Sequence comparison PFS2/QoL/PAin (A vs B)

Final Design

CRYODESMO-02 is coming

- CRYODESMO-02 will be funded by the French National Cancer Institute
- To an unprecedented amount of almost 800.000 euros
- A nationwide effort involving all GSF centers and SOS-Desmoide to promote cryoablation as a breakthrough in the care of DT patients

Conclusions

- Importance of the SOS DESMOIDE patients advocacy group as a strong support for research in France, including academic projects submitted to the French National Cancer Institute
- Strong and fruitful interactions with the French Sarcoma Group, leading to shared expertise and common projects, with patients' inputs particularly on QoL issues
- A mutually beneficial experience that is exemplary