



# GENE THERAPY

DR. JULIE KANTER  
GENE THERAPY  
TRIALS IN PROGRESS

## WHY

1PT MUTATION DIFFERENCE

B/W

**NORMAL HB**  
FLEXIBLE, OXYGEN RICH

**SICKLED**  
RIGID, OXYGEN DEFICIENT CELL

→ **CURE SUCCESS**  
MEASURED 3 WAYS

IF WE CAN EXCHANGE THE MUTATED GENE FOR THE RIGHT ONE...

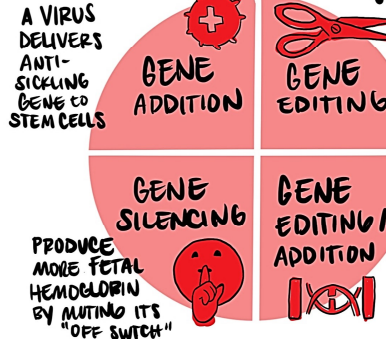
- IMPROVE HB FUNCTION
- REDUCE SCD SYMPTOMS

**BIOLOGIC**  
SICKLING STOPPED  
HEMOLYSIS RESOLVED

**FUNCTIONAL**  
REDUCED PAIN  
↓  
FATIGUE

## HOW

### 4 TYPES OF GENE THERAPY



**EFFICACY**  
MEASURED BY  
VCN % OF TRANSPOSED BLOOD STEM CELLS  
CELL DOSAGE

## RISK

- RANDOM VECTORS
- CANCER
- CHEMO-RISK
- STEM CELL ABNORMALITIES OR DELETION
- OFF TARGET EFFECTS

**FUTURE** OF GENE THERAPY

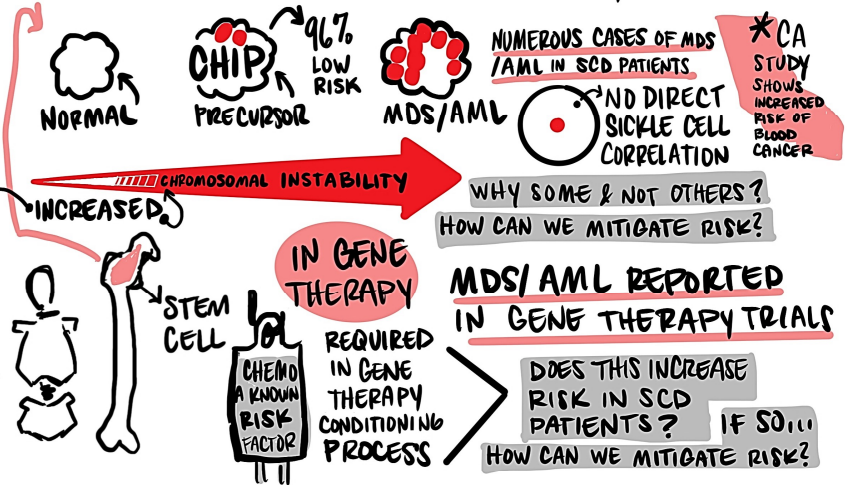
GRADE OF CURE RISK & OUTCOME TRANSPARENCY ACCESSIBILITY OF CARE MONITORING LONG-TERM EFFECTS

## DR. LACHELLE WEEKS SICKLE CELL DISEASE (SCD) GENE THERAPY & LEUKEMIA

**RISK for MDS/AML → ACQUIRED**

- AGE (biggest factor)
- ENVIRONMENTAL EXPOSURE
- CHEMO-RADIATION
- RARE SYNDROMES

MUTATIONS & ABNORMAL GROWTH OF BLOOD STEM CELLS



## PANEL PERSPECTIVE

MODERATED BY DEREK ROBERTSON

**RYAN COOPER**  
SCD PATIENT & PEER ADVOCATE  
HAPPY TO BE HERE

**RONISHA EDWARDS-ELLIOT**  
SCD PATIENT-ADVOCATE  
I WANT TO KNOW MORE ABOUT GENE THERAPY & RISK -VS- BENEFIT

**SHAVNA WHISENTON**  
LEARNING MORE ABOUT UNDERLYING CAUSES  
REQUIREMENTS FOR TRIAL EXCLUSION

**WALLY SMITH**  
GEN INTERNIST  
MOST CONSULT FAMILY & FRIENDS  
TRUST GAP W/IN MINORITY COMMUNITIES

**NANCY**  
CARE GAPS & MEDICAL ACCESS  
LACK OF PRIMARY CARE

PATIENTS NEED MEDICAL A HOME

WE ARE ALL LEARNING...  
INFO BROKERS ARE CRITICAL



**HOW DO WE SPEAK TO PATIENT FEARS?**  
BE FULLY TRANSPARENT TELL THE GOOD, BAD & UGLY UNDERSTAND WE ARE PEOPLE ADDRESS FEARS & STIGMAS

**HOW WILL TREATMENTS BECOME ACCESSIBLE?**  
LEAN ON YOUR CARE PHYSICIAN HOW DID YOU DECIDE TO GO THROUGH W/ IT? SUCCESS STORIES HAVING A MEDICAL HOME

**LOCATE TRIALS**  
COMPETITION YES VC FOUNDATION GOOD FOR INNOVATION

**WHY WERE TRIALS RE-OPENED?**  
IT WASN'T THE VECTOR SAFER... MORE PRECAUTION IN PROCEDURE

**CALIFORNIA STUDY WAS RETROACTIVE A BACKWARDS LOOK**

**RELATIVE VS ABSOLUTE RISK**  
0-2% vs 0-30%

**INVISIBLE NEEDLE IN A HAYSTACK**  
DON'T KNOW WHAT WE'RE LOOKING FOR... YET.

**SOME AREAS MORE DATA RICH**

**NO FORMAL SCREENING CURRENTLY**  
CAN ONLY DETECT LESIONS

**96% w/ CHIP DO NOT develop Cancer**

**DO ALL THERAPIES REQUIRE CHEMO?**  
CURRENTLY YES CLEAN OUT DAMAGED CELLS HARVEST NEW LESS CHEMO IS MORE RISKY

**MORE TRAINING FOR MEDICAL INFO BROKERS**  
SURVEILLANCE DATA PROXIMITY IS KEY CELLS WANT SURVIVE TRIP SIMULTANEOUS STUDIES IN EUROPE COLLABORATE BY SHARING DATA

**HIGH LEVEL DR. VISITS EVIDENCE OF TEST ORDERS**

see in **Colors**