GENE THERAPY

WHAT

FETAL HEMOGLOBIN

WHY

IMPROVING HB FUNCTION

1 PT MUTATION DIFFERENCE

SUCCESS MEASURED 3 WAYS

SUCCESS

HOW

4 TYPES OF GENE THERAPY

HERE CAN WE EXCHANGE THE MUTATED GENE FOR THE RIGHT ONE

IF WE CAN EXCHANGE THE MUTATED GENE FOR THE RIGHT ONE

GENE ADDITION

A VIRUS DELIVERS ANTI-SICKLING GENE TO STEM CELLS

GENE EDITING

CHEMICALLY CUTS OUT MUTATION PASTES NEW GENE

GENE SILENCING

PRODUCE MORE FETAL HEMOGLOBIN BY MUTATING IT "OFF SWITCH"

GENE ADDITION

EDIT THE GENE OCCURRENCE & INITIATE GENE REPAIR PROCESS

HOW

GENE

RISK

RANDOM VECTORS

CANCER

CHEMO-REBOUND

STEM CELL ABNORMALITIES

OFF TARGET EFFECTS

FUTURE

GRADE 4 CURE

TRANSIENT ACCESSIBILITY OF CARE

CA

STUDY SHOWS INCREASED RISK OF BLOOD CANCER

DOMINO-RESISTANCE

MORE CASES OF MDS/AML IN SCD PATIENTS

RISK FOR MDS/AML → ACQUIRED MUTATIONS

NODULES & AML

AGGREGATION & GROWTH OF CHEMORADIATION

RARE SYNDROMES

RISK FOR MDS/AML → ACQUIRED MUTATIONS

ABILITY OF GENE

THIRDARY

LEUKEMIA

LEUKEMIA

DR. LACHELLE WEEKS

SICKLE CELL DISEASE (SCD)

GENE THERAPY & LEUKEMIA

RISK FOR MDS/AML → ACQUIRED MUTATIONS

NORMAL

CHIP PRECURSOR

MDS/AML

AND DIRECT SICKLE CELL CORRELATION

WHY SOME & NOT OTHERS?

HOW CAN WE MITIGATE RISK?

MDS/AML REPORTED IN GENE THERAPY TRIALS

REQUIRE DIRECT GENE THERAPY TO CONDITIONING PROCESS

THIRDARY EFFECTS

DOES THIS INCREASE RISK IN SCD PATIENTS?

IF SO...

HOW CAN WE MITIGATE RISK?

GENE THERAPY

PANEL PERSPECTIVE

FUTURE

MODERATED BY DEREK ROBERTSON

RAN COOPER

SCD PATIENT & PEER ADVocate

WHY IS IT SO HAPPY TO BE HERE?

RONISHA EDWARDS-ELLIOT

SICKLE CELL PATIENT + ADvocate

I WANT TO KNOW MORE ABOUT GENE THERAPY & RISK- Vs- BENEFIT

SHARON WHITSON

LEARNING MORE ABOUT UNDERLYING CAUSES

MATHEMATICAL REQUIREMENTS FOR TRIAL ELUSION

NANCY

CHEMOSAPS & MEDICAL ACCESS

LACK OF PRIMARY CARE

PATIENTS NEED MEDICAL HOME