



Spinal Muscular Atrophy and Current Trends

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-CHARLOTTE HOLLMAN, MD

EXCITING DEVELOPMENTS

COOLING FOR HYPOXIC BABIES

NUSINERSIN NEW!

- \$750K FOR 1ST YEAR + \$375K EVERY YR FOLLOWING
- NOT A LOT OF IMPROVEMENT

ALL FORMS OF S.M.A. ARE SEVERE

OMNASEMNOGENE ABEPARVOVEC

- GENE THERAPY LIMITED TO UNDER 2
- \$2.1 M FOR 1 DOSE & REST OF LIFE



SMA 1 MOST COMMON AND PRESENTS IN INFANCY

AUTO IMMUNE TREATMENT

"GENE THERAPY FIRST"

SMA 2 WILL SIT AT SOME POINT

GENE THERAPY

RISDIPLAM

- ONCE DAILY / LIFE

SMA 3 NORMAL LIFE EXPECTANCY

SMA 4 LEAST SEVERE RARE ADULT ONSET
SCOUZIS BREATHING ISSUES

