Dr. P Gurugama Video 2: Rationale for CRISPR-Based Therapies: Unmet Needs in Standard of Care Treatments Transcript

00:05 - 00:13

While there are treatments available for many chronic diseases today, managing disease symptoms and progression can mean a lifetime commitment to therapy and chronic dosing.

00:14 - 00:37

This can come with significant administration and financial burdens for patients.

While some patients show improvements and slowed disease progression with standard of care treatments, others may experience incomplete responses.

Incomplete response may occur because most standard of care therapies target disease mechanisms at the protein and RNA levels, which may not sufficiently reduce disease activity.

00:38 - 00:50

Since current standard of care therapies may not sufficiently address the underlying cause of disease, this unmet treatment need can leave many patients vulnerable to persistent symptoms and ongoing disease progression.

00:51 - 01:04

I once had a patient with a debilitating lifelong condition who received long-term standard of care treatments. Yet they had intermittent breakthrough symptoms that were totally unpredictable. There was a clear unmet need.

01:05 - 01:12

Gene editing approaches have the potential to durably address underlying disease activity, potentially eliminating the need for chronic therapy.

01:13 - 01:26

Intellia designed their investigational *in vivo* CRISPR/Cas9 gene editing therapies with this in mind and is evaluating the safety and efficacy of these therapies as a one-time treatment in late-stage clinical trials.