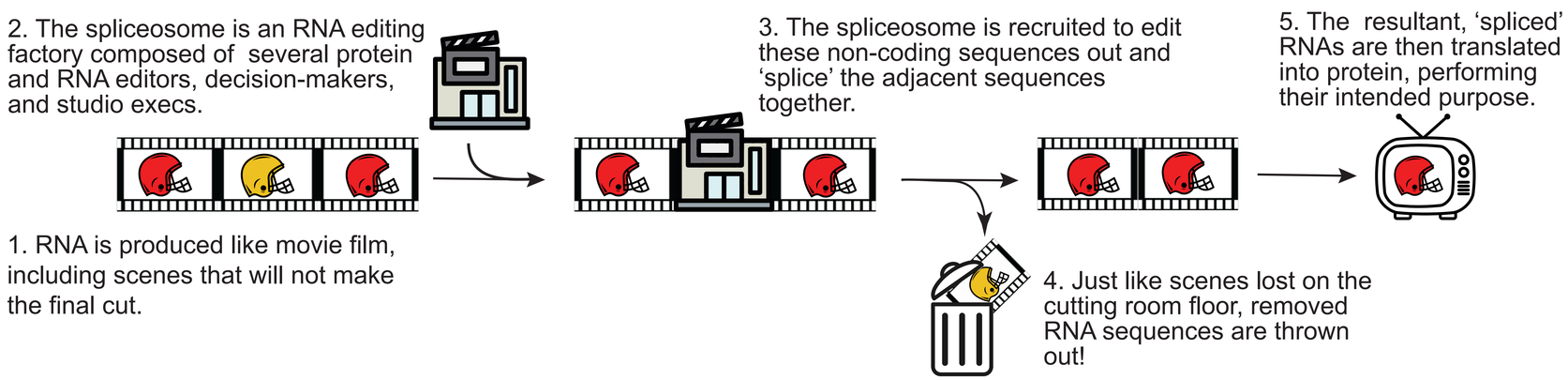
Splicing: Critical for Good Movies and Good Health

Anton Blatnik[1,2] and Caleb Embree[2,3]

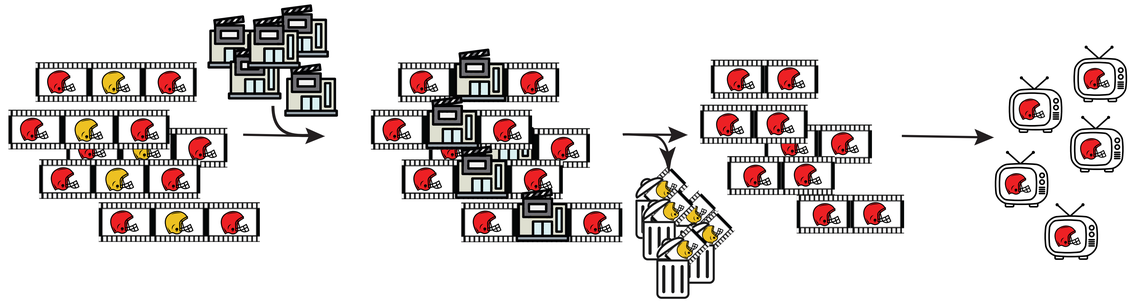
1: Biochemistry & Pharmacology, The Ohio State University College of Medicine 2: Center for RNA Biology, 3: Molecular Genetics, Ohio State University

In the final cut, unwanted parts of movies are removed or spliced out. Similarly, segments of newly made RNAs are spliced together in order to make proteins in appropriate amounts. In diseases like Spinal Muscular Atrophy (SMA), misfiring of the cellular "editing machine" leads to faulty RNAs and dysfunctional proteins. At OSU, we have developed therapies for SMA that restore function of cellular editors to ensure correct splicing of RNAs.

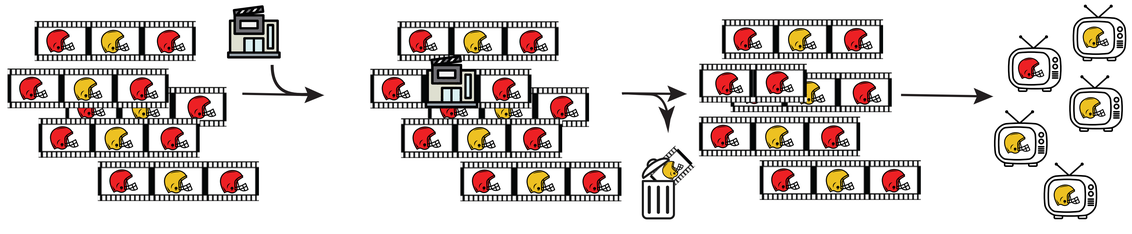


The spliceosome is a large and complex machine. Mutations that affect this machine have been shown to cause multiple human diseases. Many of these disrupt development of the head and nervous system, resulting in neurodegenerative diseases like Spinal Muscular

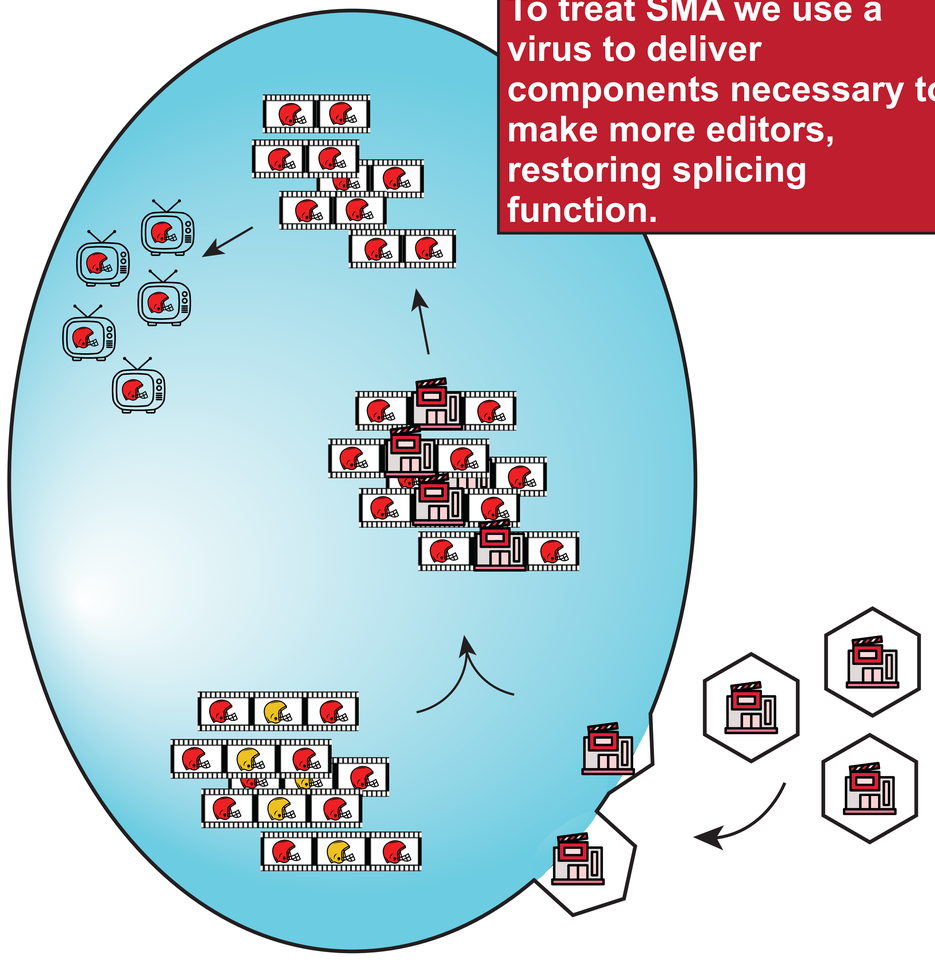
Usually, there are enough editors to keep up with the number of films being created, resulting in polished final features:



In SMA there are not enough editors to keep up with the demand of films being produced resulting in 'unfinished' or unedited movies:



To treat SMA we use a virus to deliver components necessary to make more editors, restoring splicing function.



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