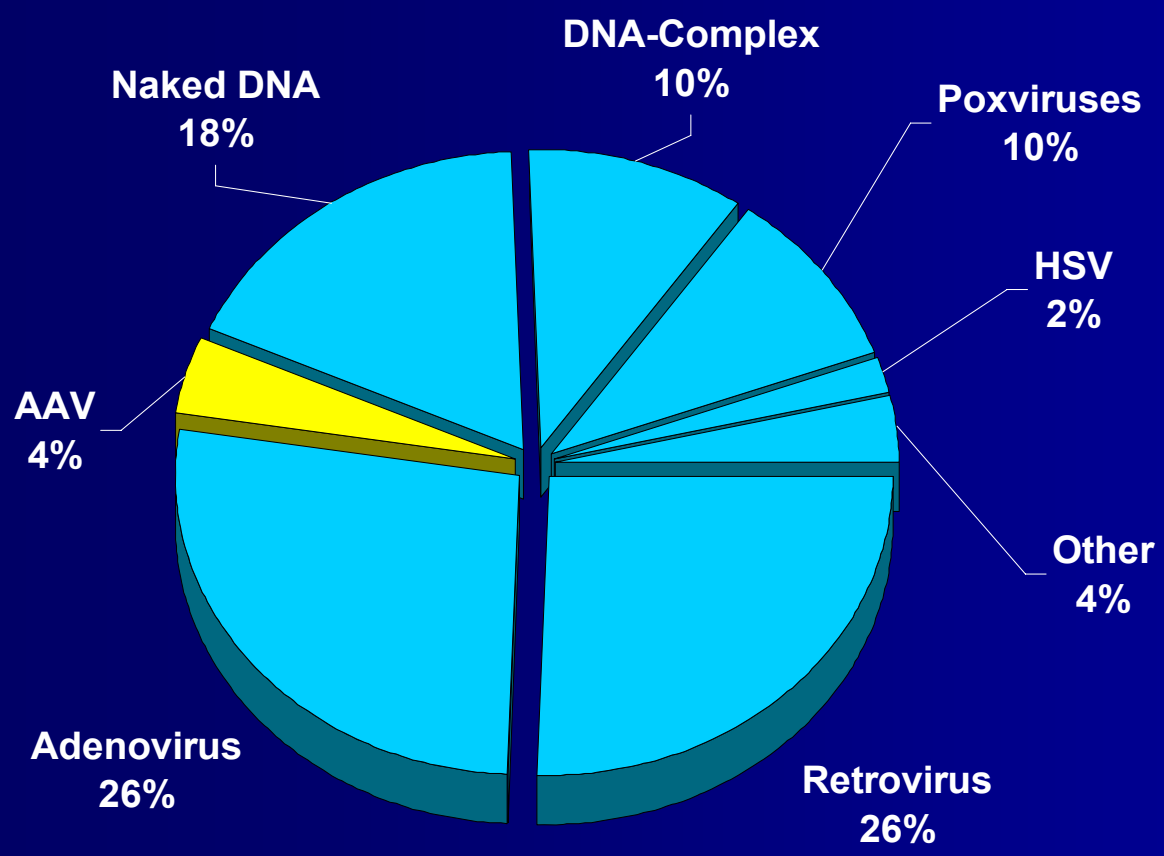
AAV Vectors for Gene Transfer

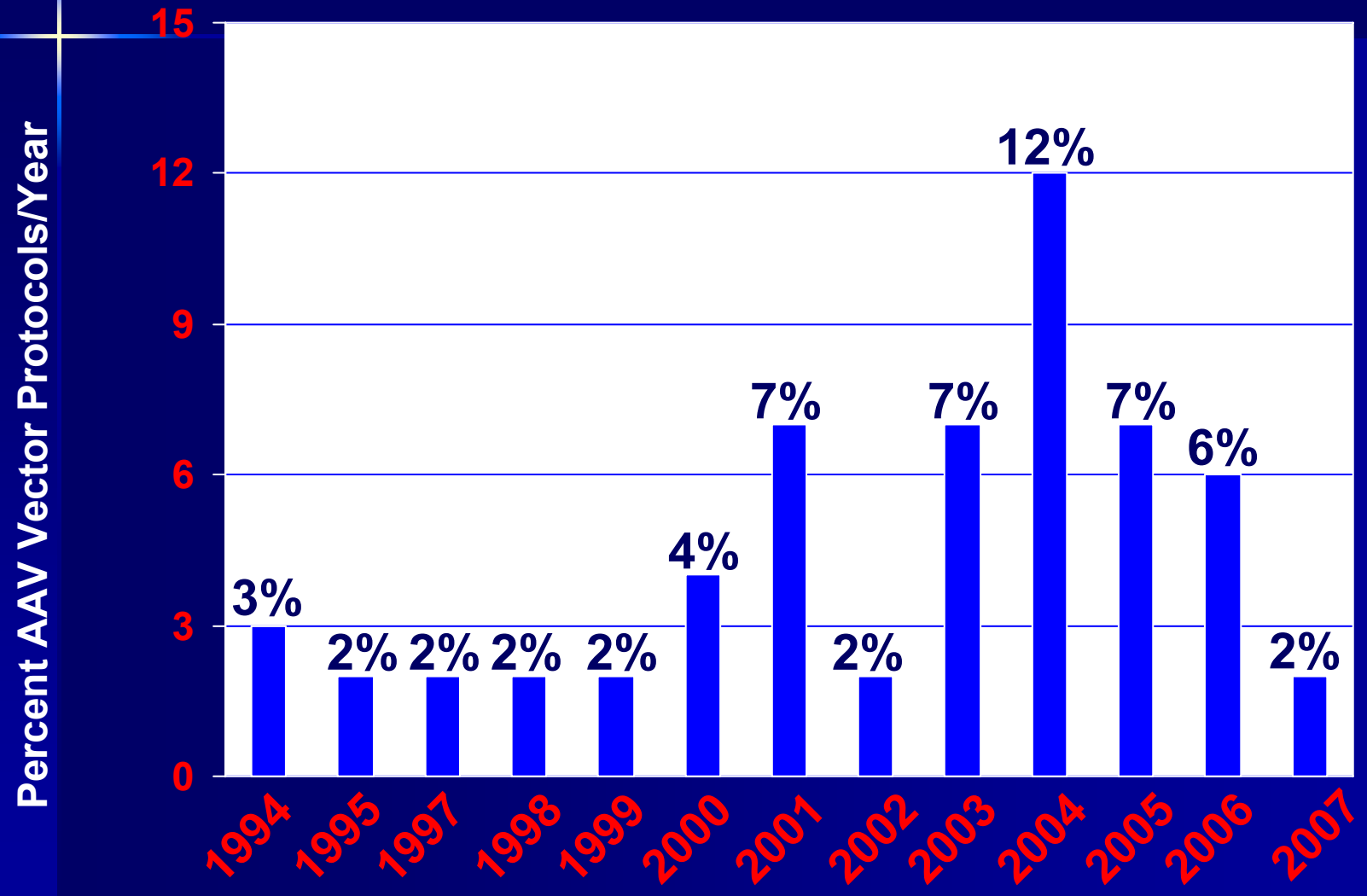
NIH Office of Biotechnology Activities
Sept 17, 2007

Gene Transfer Trials By Delivery System

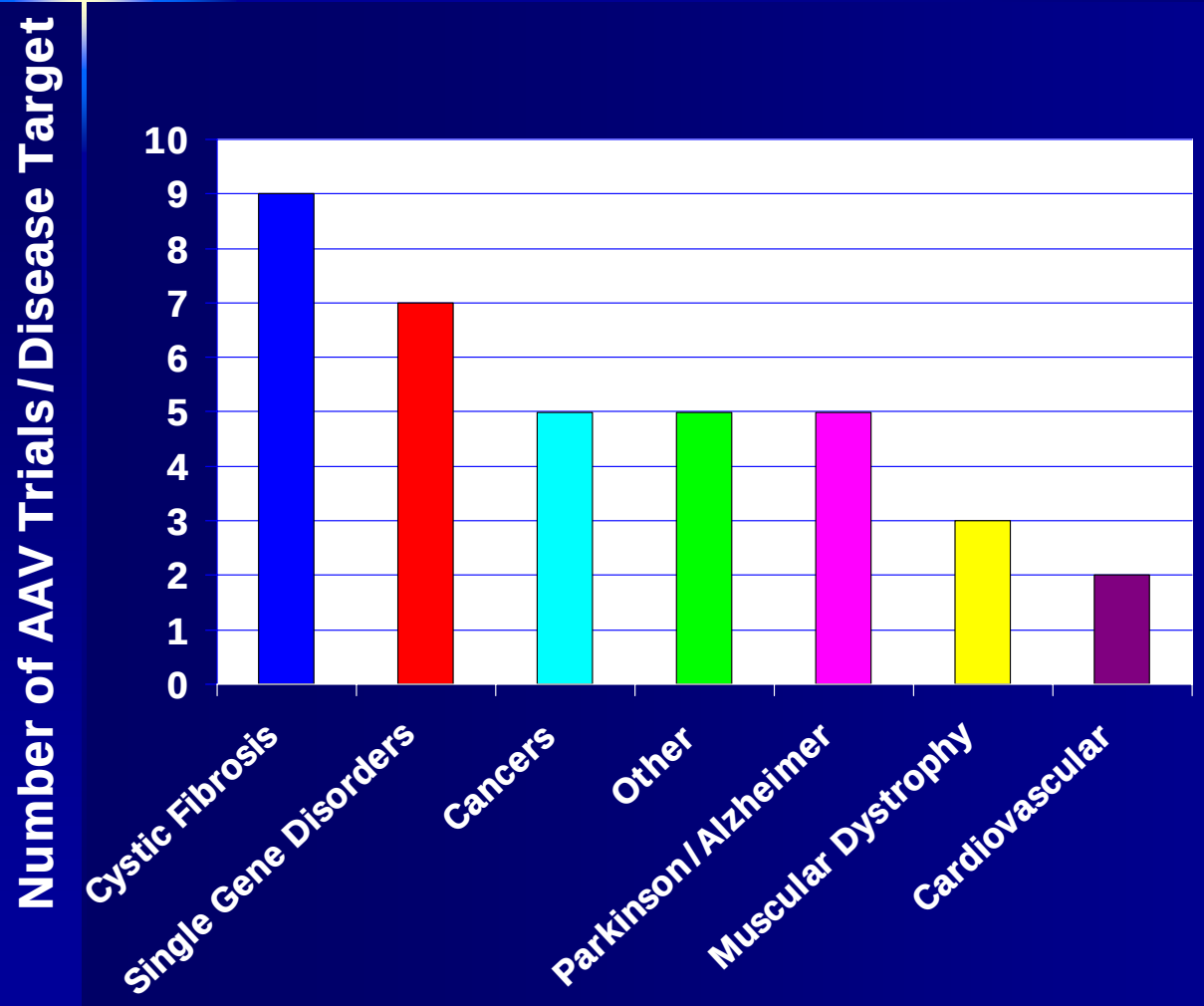


Total Trials = 871
AAV Trials = 36
AAV2 = 30
AAV2.5 = 1
AAV1 = 3
AAV6 = 1
AAV8 =1

Protocols Using AAV Vectors as a Percentage of Total Protocols Submitted to OBA by Year



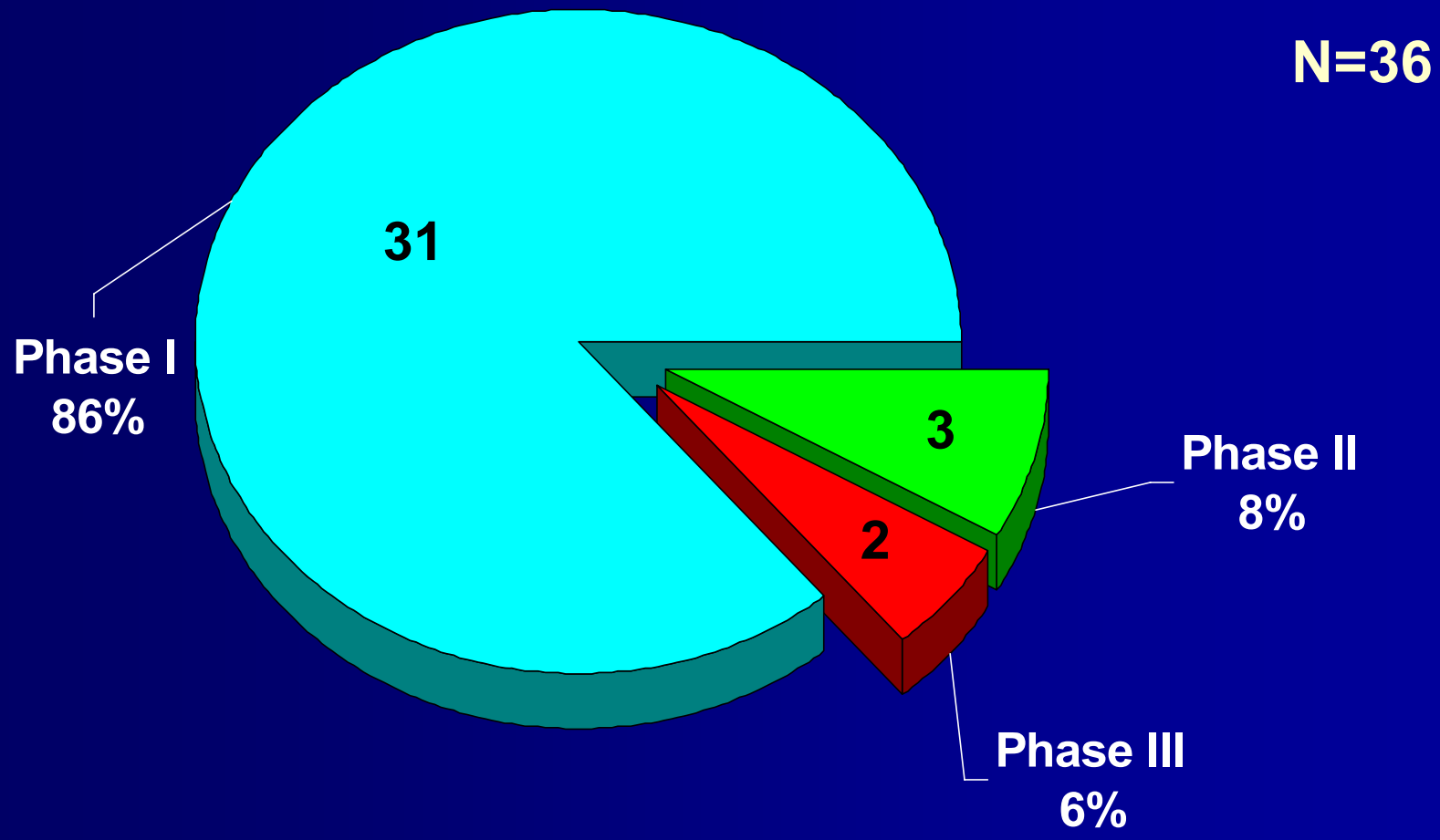
Target disease for AAV Trials



Single Gene Disorders:
- Hemophilia B
- Canavan Disease
- α-1-anti-trypsin deficiency
- Late infantile neuronal ceroid lipofuscinosis

Other:
- Retinal disease
- Epilepsy
- Arthritis

AAV Gene Transfer Trials by Phase



Rev: 5/2/07/ts

Serious Adverse Events on AAV Trials

- GeMCRIS was used to identify 34 initial adverse event reports from thirteen trials that were both *unexpected* and *possibly related* to the gene transfer with an AAV vector
- All of these events have been routinely reviewed by the Gene Transfer Safety Assessment Board
- A review of these events did not identify any patterns across AAV trials
- The types of serious adverse events observed in AAV trials did not seem to differ from those observed in other gene transfer trials.