

## Adeno-Associated Virus Type 2 Reference Standard Material is Now Available

The AAV2 vector reference standard material (an AAV2-GFP viral vector) is now available from ATCC (<http://www.atcc.org/>). The AAV2 RSM is intended for use in calibrating internal (laboratory-specific) reference materials and assays for recombinant AAV viral gene transfer products, with the purpose of making data from different pre-clinical and clinical studies more comparable.

Both the AAV2 RSM and the pTR-UF-11 vector plasmid used to produce the RSM are now available from ATCC for a nominal cost (product numbers [ATCC VR-1616™](#) and [ATCC MBA-331™](#) respectively). The cell line, HeLa RC32 ([ATCC CRL-2972™](#)), and the adenovirus type 5 helper virus ([ATCC VR-1516™](#)) used to conduct AAV2 RSM infectious titering are also available. Each vial of the AAV2 RSM contains 0.5 mL containing  $3.28 \times 10^{10}$  vg/ml ( $=4.37 \times 10^9$  TCID<sub>50</sub>IU/ml).

With an NIH NCRR grant and donations from industry and numerous academic organizations, the AAV2 RSM was produced and purified at the Vector Core of the University of Florida's Powell Gene Therapy Center, and the vials were filled and stored in the repository at ATCC. Characterization of the AAV2 RSM was performed by 16 laboratories worldwide.

In May of 1999 at a joint FDA/NIH workshop, members of the rAAV gene therapy community from academia, industry, and the federal government discussed the value of sharing a body of data to address vector-related safety issues (please refer to the Bioprocessing Journal website: [www.bioprocessingjournal.com](http://www.bioprocessingjournal.com)). It was generally accepted that sharing and comparing pre-clinical and clinical data developed with different vector-transgene combinations would be valuable for determining vector dose, strength, and potency in terms of equivalent titer units. As a result, it was agreed that a highly characterized rAAV reference material would be needed to facilitate these comparisons and allow researchers to normalize their titer values.

In the United States, the FDA Center for Biologics Evaluation and Research (CBER), Office of Cellular, Tissue, and Gene Therapies (OCTGT), Division of Cellular and Gene Therapies (DCGT) recommends reference materials as benchmarking tools for qualifying and validating “in house” reference standards and assays by comparison to the collective AAV2RSM data. It should be noted that it is not the intent of the FDA to standardize assay methods across the field or to require that the values assigned to the rAAV2RSM be duplicated during validation studies. Furthermore, there is no requirement in the United States to follow rAAV2 RSM procedures when assaying particle concentration, genome copy number or infectious titer. Sponsors of adeno-associated virus-related INDs should consult with the FDA/CBER or appropriate national agency for further guidance.

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