

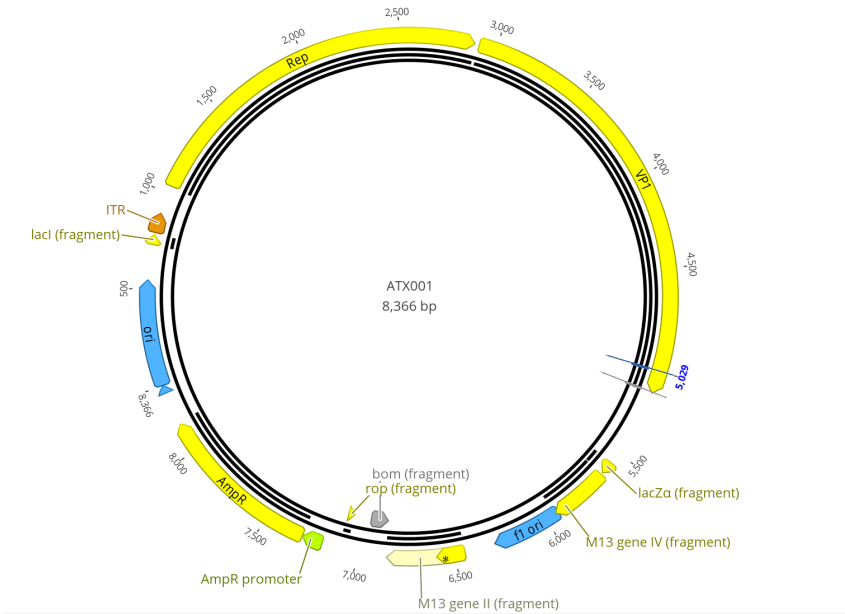
AAV Batch #: ATX001-20241115-scCAG-GFP

Serotype: ATX001

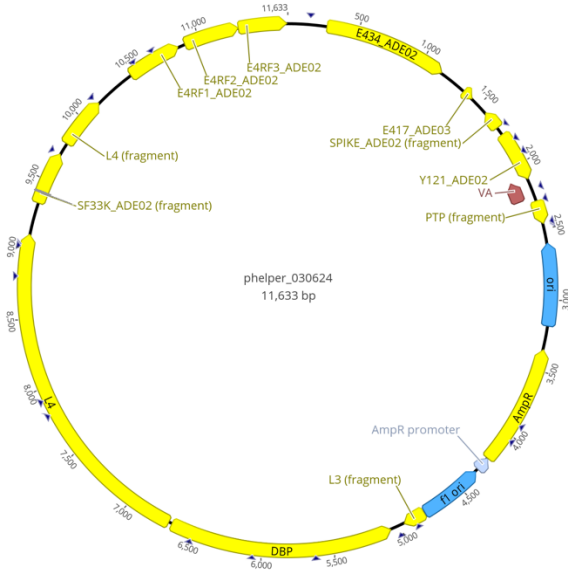
AAV construct: pAAV-scCAG-GFP

Plasmid map:

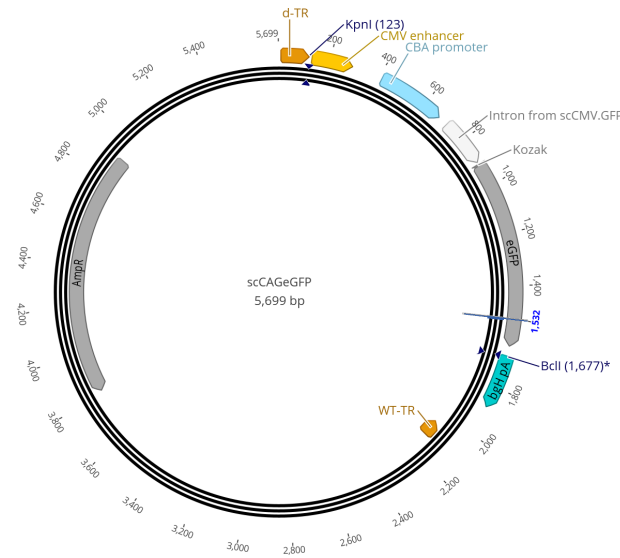
ATX001(rep/cap):



pHelper:



- scCAG-GFP-BC226:
- scCAG-GFP-BC234:
- scCAG-GFP-BC247:
- scCAG-GFP-BC231:
- scCAG-GFP-BC227:
- scCAG-GFP-BC228:
- scCAG-GFP-BC232:



Packaging protocol and Titering:

Barcoded AAVs were purchased commercially from Packgene and diluted in-house. The titers were measured using qPCR AAV titer kit (ABM, catalog# G931), following the manufacturer's protocol.

AAV construct	Titer (Vg/mL)
ATX001-CAG-BC226- GFP	1.00e+13
ATX001-CAG-BC234- GFP	1.59e+12
ATX001-CAG-BC247- GFP	2.83e+11
ATX001-CAG-BC231- GFP	1.34e+10
ATX001-CAG-BC227- GFP	2.10e+9
ATX001-CAG-BC228- GFP	1.34e+8
ATX001-CAG-BC232- GFP	1.60e+7
Final ATX001 library pool	4.08E+12

Endotoxing levels:

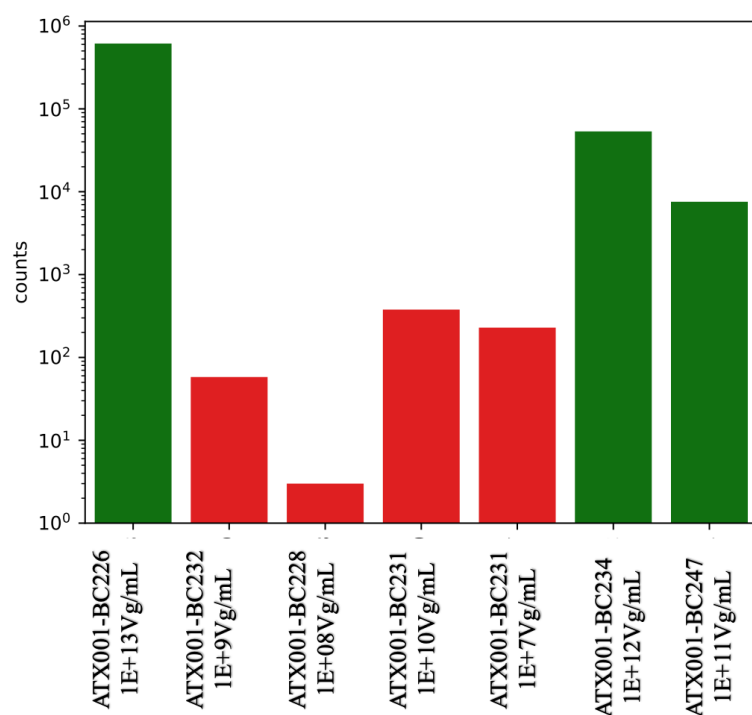
Bacterial endotoxin was detected by gel-clot method. The sample is diluted with water for BET to the maximum dilution factor. Take the sample dilution and mix it with an equal volume of LAL reagent (e.g., 0.1 ml aliquot). Continue incubating the reaction mixture (at 37 ± 1 °C for 60 ± 2 min) without vibration. To test the integrity of the gel, remove each tube directly from the incubator and invert it through approximately 180° in one smooth motion. If a firm gel is formed that remains in place upon inversion, record the result as positive. If no intact gel is formed, the result is negative.

AAV construct	Value (EU/mL)
ATX001-CAG-BC226- GFP	<0.2

ATX001-CAG-BC234- GFP	<0.2
ATX001-CAG-BC247- GFP	<0.2
ATX001-CAG-BC231- GFP	<0.2
ATX001-CAG-BC227- GFP	<0.2
ATX001-CAG-BC228- GFP	<0.2
ATX001-CAG-BC232- GFP	<0.2
Final ATX001 library pool	<0.5

Dilution curve analysis:

Final titer of the pooled dilution curve was 4.08E+12 vg/mL. Barcodes from the pooled library were amplified by PCR and then subjected to sequencing on an Illumina iSeq100. Quantification of barcodes revealed the following library balance:



QC provided by Packgene is copied below.

Certificate of Analysis (COA)

Name	ATX001 (Engineered AAV2, contains loop insertion)[CAG-GFP.Barcode 226]	Lot#	19347-2T
Order#	USJW241008942-14	Date	2024-11-15

Items	Analytical Method	QC results
[Genome Titer]	qPCR	Adjusted Titer: 1E+13GC/mL
[Purity]	SDS-PAGE	No other significant band
[Endotoxin]	LAL	<0.2EU/mL

Conclusion:Qualified

* Adjusted Titer: Titers adjusted to order requirements.

Item 1: Genome Titer

Lot#	Quantity Mean GC/ml	Calibration Factor	Dilution factor*	Detected Titer(GC/mL)	Adjusted Titer (GC/mL)
19347-2T	1.49E+12	0.52	20	1.54E+13	1E+13

* Reference AAV:Packgene:AAV2[ssAAV.CAG.EGFP.WPRE.SV40pA],Labeled titer: 5.5E+11(GC/mL)

Figure 1: Sample Location

	1	2	3	4	5	6	7	8	9	10	11	12
A	ITR 8E14	ITR 8E14	ITR 8E14			19347-2 ITR 1.52E12	19347-2 ITR 1.46E12					
B	ITR 8E13	ITR 8E13	ITR 8E13									
C	ITR 8E12	ITR 8E12	ITR 8E12									
D	ITR 8E11	ITR 8E11	ITR 8E11									
E	ITR 8E10	ITR 8E10	ITR 8E10									
F	ITR 8E9	ITR 8E9	ITR 8E9									
G	Ref AAV ITR 1.11E12	Ref AAV ITR 1.03E12	Ref AAV ITR 1.06E12									
H	NC ITR 3.15E8	Dnase ITR 8.55E7										

Figure 2 Amplification curves

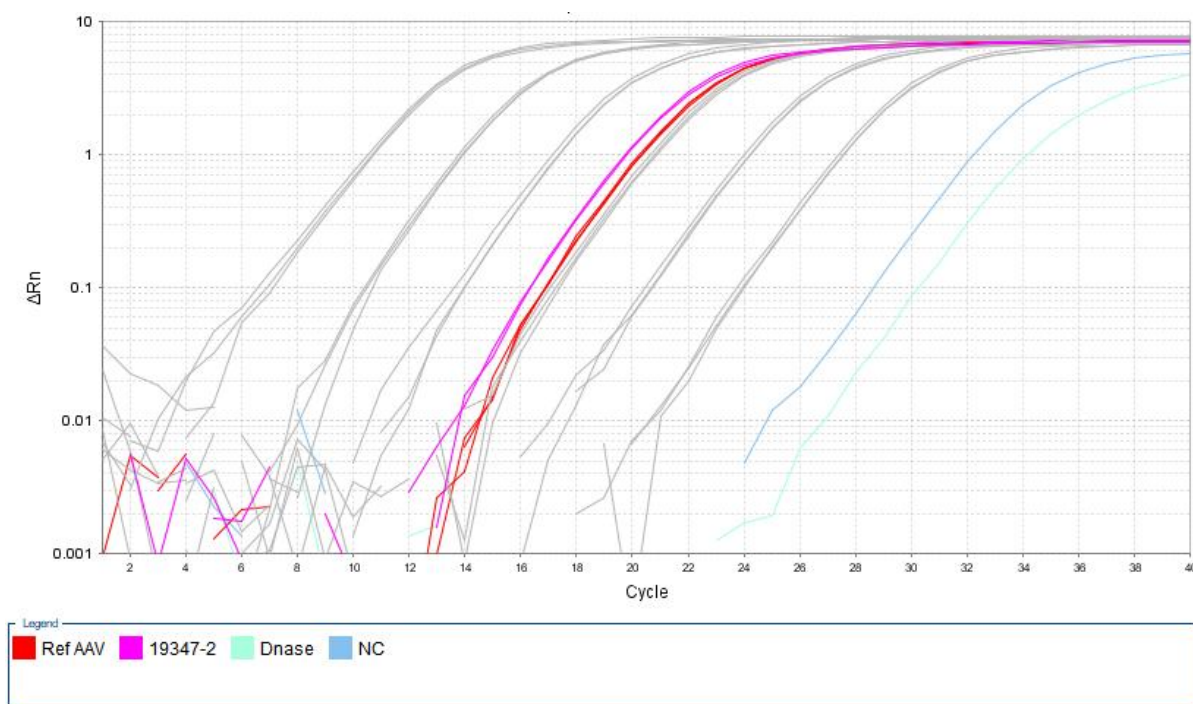
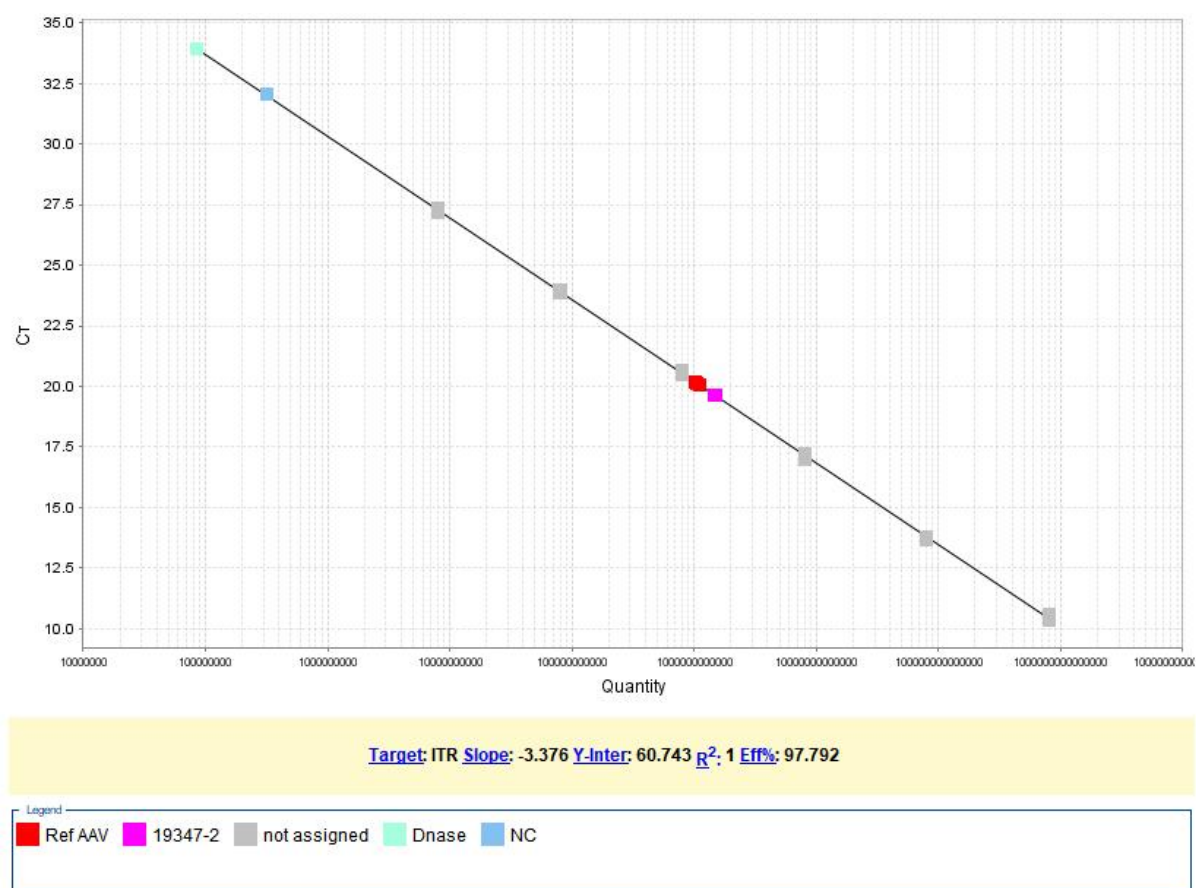


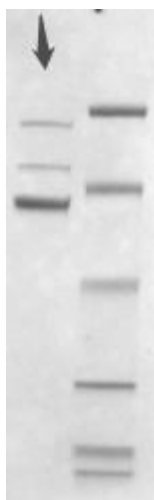
Figure 3 Standard Curve



Item 2: Endotoxin

Name	Sample positive control	Positive control	Neg control	Sample
Result	+	+	—	—

Item 3: Purity (SDS-PAGE)



Maker:97.2KD,66.4KD,44.3KD,29KD,20.1KD,14.3KD

Conclusion: No other significant band

Certificate of Analysis (COA)

Name	ATX001 (Engineered AAV2, contains loop insertion)[CAG-GFP.Barcode 227]	Lot#	19348-2E1
Order#	USJW241008942-16	Date	2024-11-15

Items	Analytical Method	QC results
[Genome Titer]	qPCR	Adjusted Titer: 3.72E+12GC/mL
[Purity]	SDS-PAGE	No other significant band
[Endotoxin]	LAL	<0.2EU/mL

Conclusion:Qualified

* Adjusted Titer: Titers adjusted to order requirements.

Item 1: Genome Titer

Lot#	Quantity Mean GC/ml	Calibration Factor	Dilution factor*	Detected Titer(GC/mL)	Adjusted Titer (GC/mL)
19348-2E1	3.60E+11	0.52	20	3.72E+12	3.72E+12

* Reference AAV:Packgene:AAV2[ssAAV.CAG.EGFP.WPRE.SV40pA],Labeled titer: 5.5E+11(GC/mL)

Figure 1: Sample Location

	1	2	3	4	5	6	7	8	9	10	11	12
A	ITR 8E14	ITR 8E14	ITR 8E14									
B	ITR 8E13	ITR 8E13	ITR 8E13			19348-2 ITR 3.64E11	19348-2 ITR 3.56E11					
C	ITR 8E12	ITR 8E12	ITR 8E12									
D	ITR 8E11	ITR 8E11	ITR 8E11									
E	ITR 8E10	ITR 8E10	ITR 8E10									
F	ITR 8E9	ITR 8E9	ITR 8E9									
G	Ref AAV ITR 1.11E12	Ref AAV ITR 1.03E12	Ref AAV ITR 1.06E12									
H	NC ITR 3.15E8	Dnase ITR 8.55E7										

Figure 2 Amplification curves

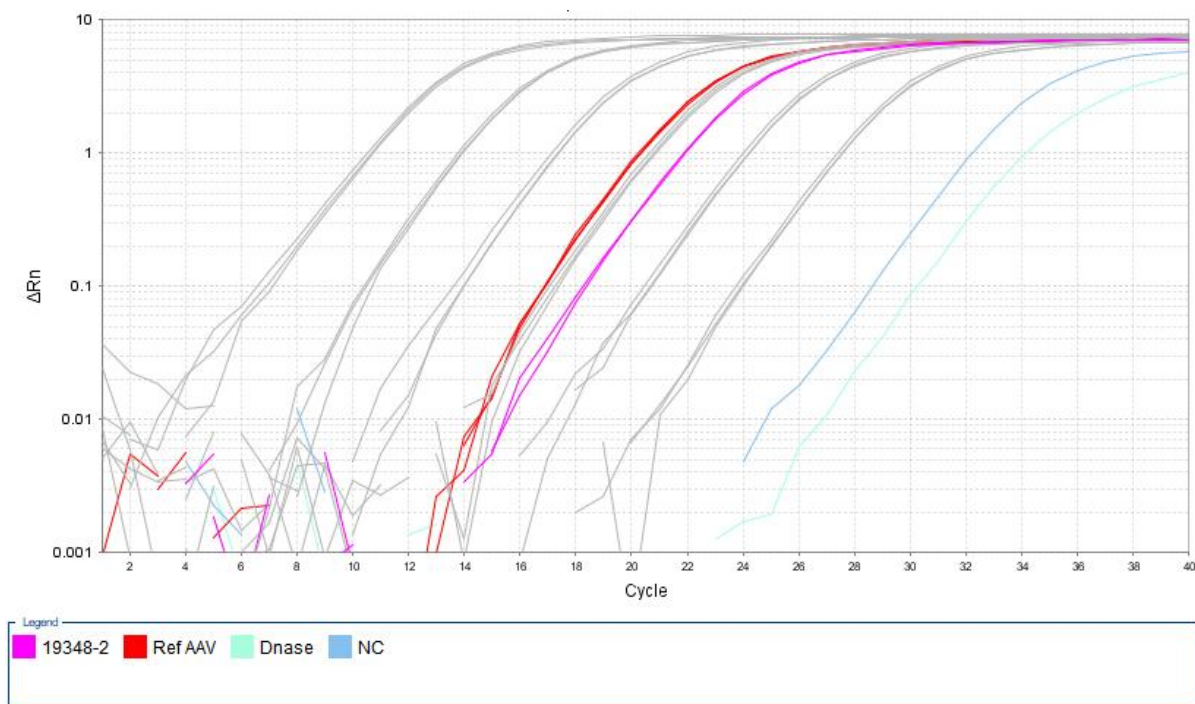
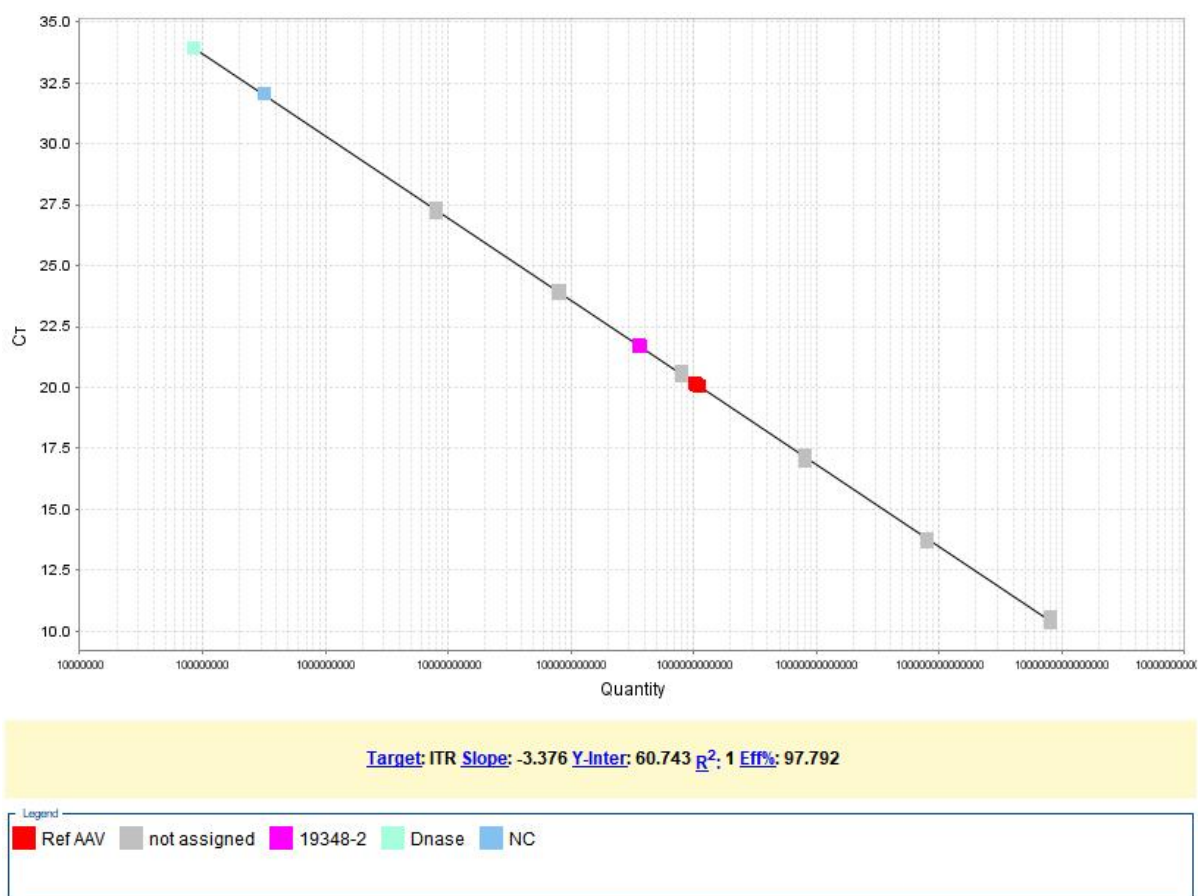


Figure 3 Standard Curve



Item 2: Endotoxin

Name	Sample positive control	Positive control	Neg control	Sample
Result	+	+	—	—

Item 3: Purity (SDS-PAGE)



Maker:97.2KD,66.4KD,44.3KD,29KD,20.1KD,14.3KD

Conclusion: No other significant band

Certificate of Analysis (COA)

Name	ATX001 (Engineered AAV2, contains loop insertion)[CAG-GFP.Barcode 228]	Lot#	19349-2Y
Order#	USJW241008942-18	Date	2024-11-15

Items	Analytical Method	QC results
[Genome Titer]	qPCR	Adjusted Titer: 3.14E+12GC/mL
[Purity]	SDS-PAGE	No other significant band
[Endotoxin]	LAL	<0.2EU/mL

Conclusion:Qualified

* Adjusted Titer: Titers adjusted to order requirements.

Item 1: Genome Titer

Lot#	Quantity Mean GC/ml	Calibration Factor	Dilution factor*	Detected Titer(GC/mL)	Adjusted Titer (GC/mL)
19349-2Y	3.04E+11	0.52	20	3.14E+12	3.14E+12

* Reference AAV:Packgene:AAV2[ssAAV.CAG.EGFP.WPRE.SV40pA],Labeled titer: 5.5E+11(GC/mL)

Figure 1: Sample Location

	1	2	3	4	5	6	7	8	9	10	11	12
A	ITR 8E14	ITR 8E14	ITR 8E14									
B	ITR 8E13	ITR 8E13	ITR 8E13									
C	ITR 8E12	ITR 8E12	ITR 8E12									
D	ITR 8E11	ITR 8E11	ITR 8E11									
E	ITR 8E10	ITR 8E10	ITR 8E10									
F	ITR 8E9	ITR 8E9	ITR 8E9									
G	Ref AAV ITR 1.11E12	Ref AAV ITR 1.03E12	Ref AAV ITR 1.06E12					19349-2 ITR 3.05E11	19349-2 ITR 3.03E11			
H	NC ITR 3.15E8	Dnase ITR 8.55E7										

Figure 2 Amplification curves

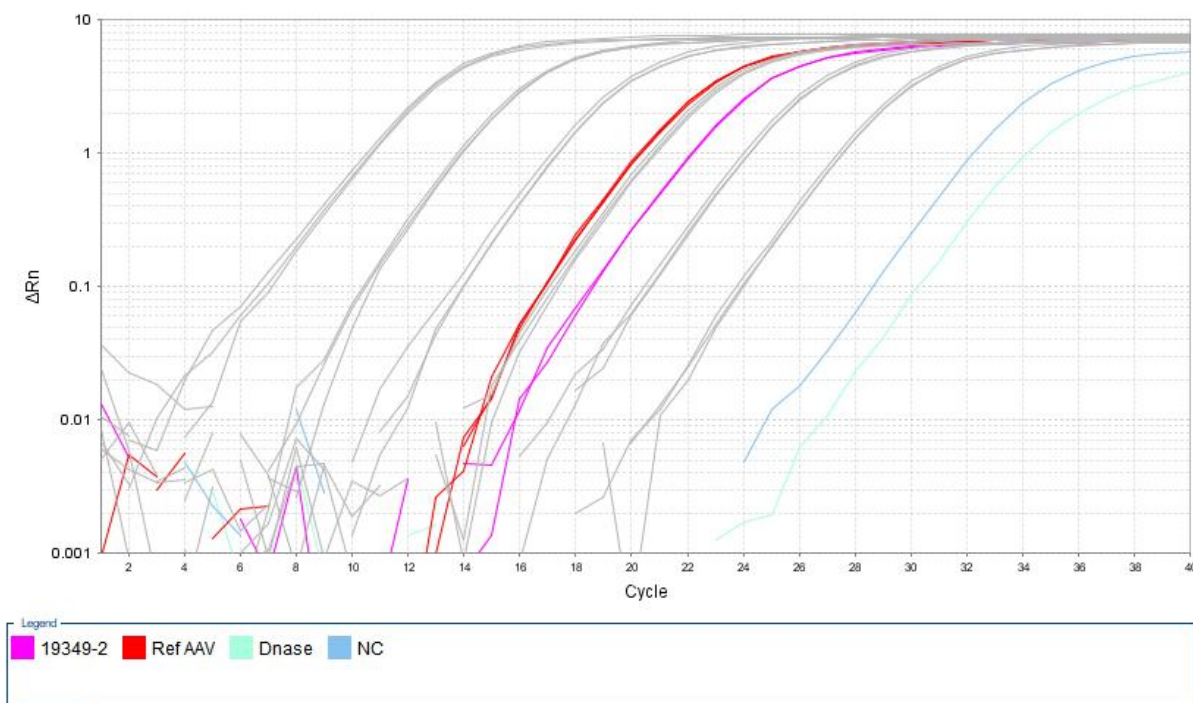
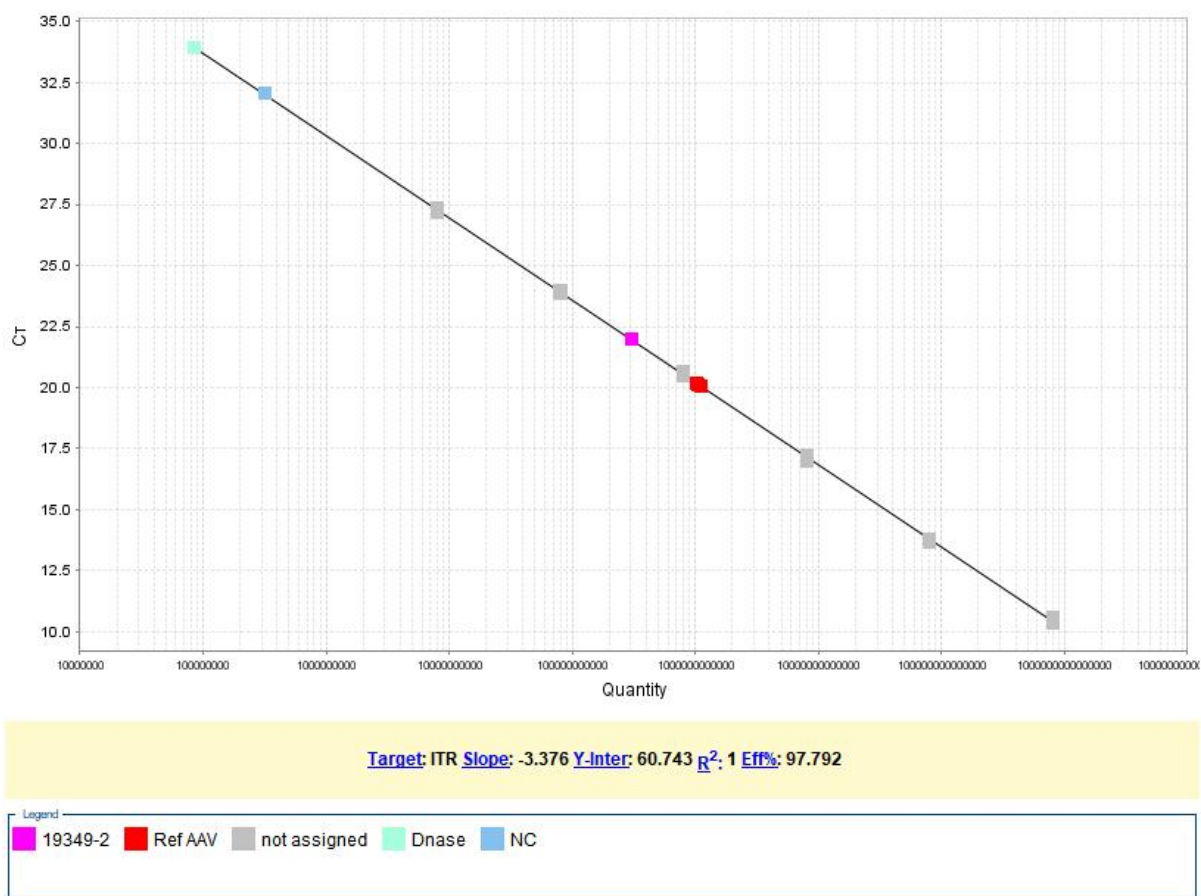


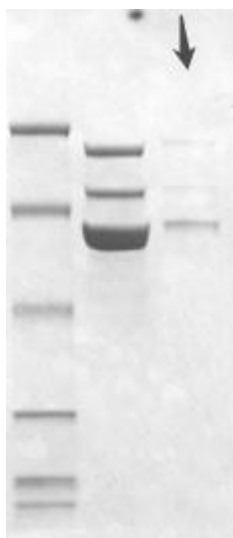
Figure 3 Standard Curve



Item 2: Endotoxin

Name	Sample positive control	Positive control	Neg control	Sample
Result	+	+	—	—

Item 3: Purity (SDS-PAGE)



Maker:97.2KD,66.4KD,44.3KD,29KD,20.1KD,14.3KD

Conclusion: No other significant band

Certificate of Analysis (COA)

Name	ATX001 (Engineered AAV2, contains loop insertion)[CAG-GFP.Barcode 231]	Lot#	19350-2E1
Order#	USJW241008942-20	Date	2024-11-15

Items	Analytical Method	QC results
[Genome Titer]	qPCR	Adjusted Titer: 3.75E+12GC/mL
[Purity]	SDS-PAGE	No other significant band
[Endotoxin]	LAL	<0.2EU/mL

Conclusion:Qualified

* Adjusted Titer: Titers adjusted to order requirements.

Item 1: Genome Titer

Lot#	Quantity Mean GC/ml	Calibration Factor	Dilution factor*	Detected Titer(GC/mL)	Adjusted Titer (GC/mL)
19350-2E1	3.63E+11	0.52	20	3.75E+12	3.75E+12

* Reference AAV:Packgene:AAV2[ssAAV.CAG.EGFP.WPRE.SV40pA],Labeled titer: 5.5E+11(GC/mL)

Figure 1: Sample Location

	1	2	3	4	5	6	7	8	9	10	11	12
A	ITR 8E14	ITR 8E14	ITR 8E14									
B	ITR 8E13	ITR 8E13	ITR 8E13									
C	ITR 8E12	ITR 8E12	ITR 8E12			19350-2 ITR 3.72E11	19350-2 ITR 3.54E11					
D	ITR 8E11	ITR 8E11	ITR 8E11									
E	ITR 8E10	ITR 8E10	ITR 8E10									
F	ITR 8E9	ITR 8E9	ITR 8E9									
G	Ref AAV ITR 1.11E12	Ref AAV ITR 1.03E12	Ref AAV ITR 1.06E12									
H	NC ITR 3.15E8	Dnase ITR 8.55E7										

Figure 2 Amplification curves

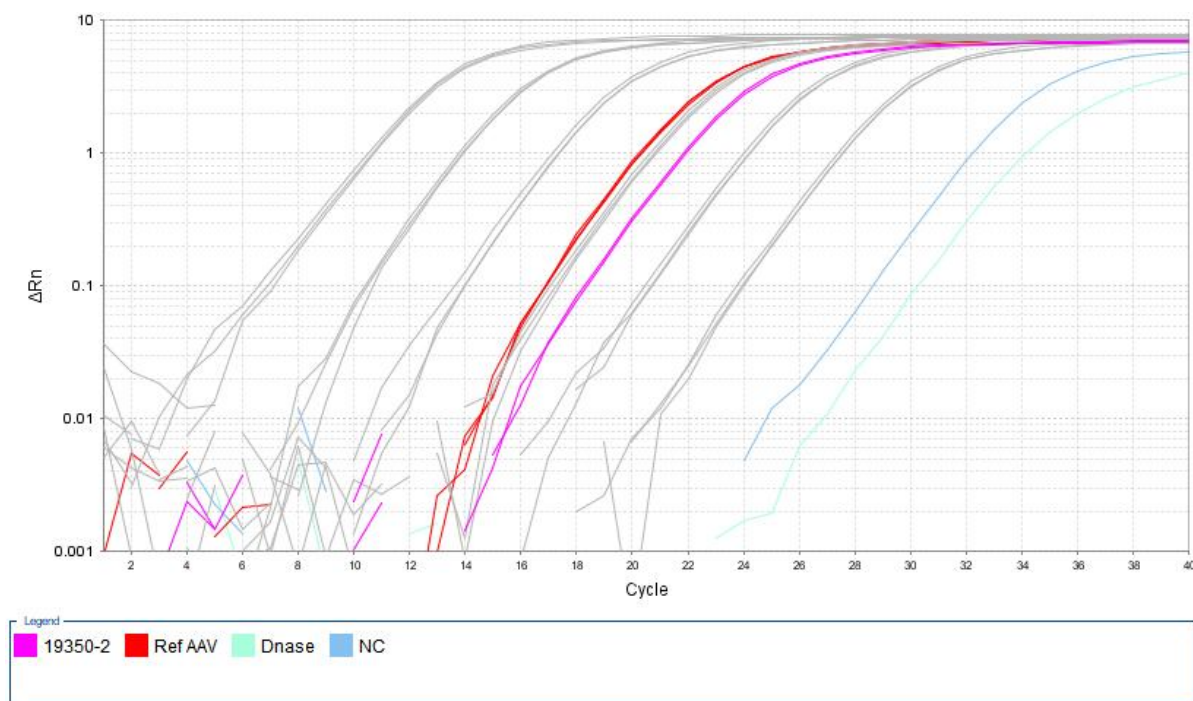
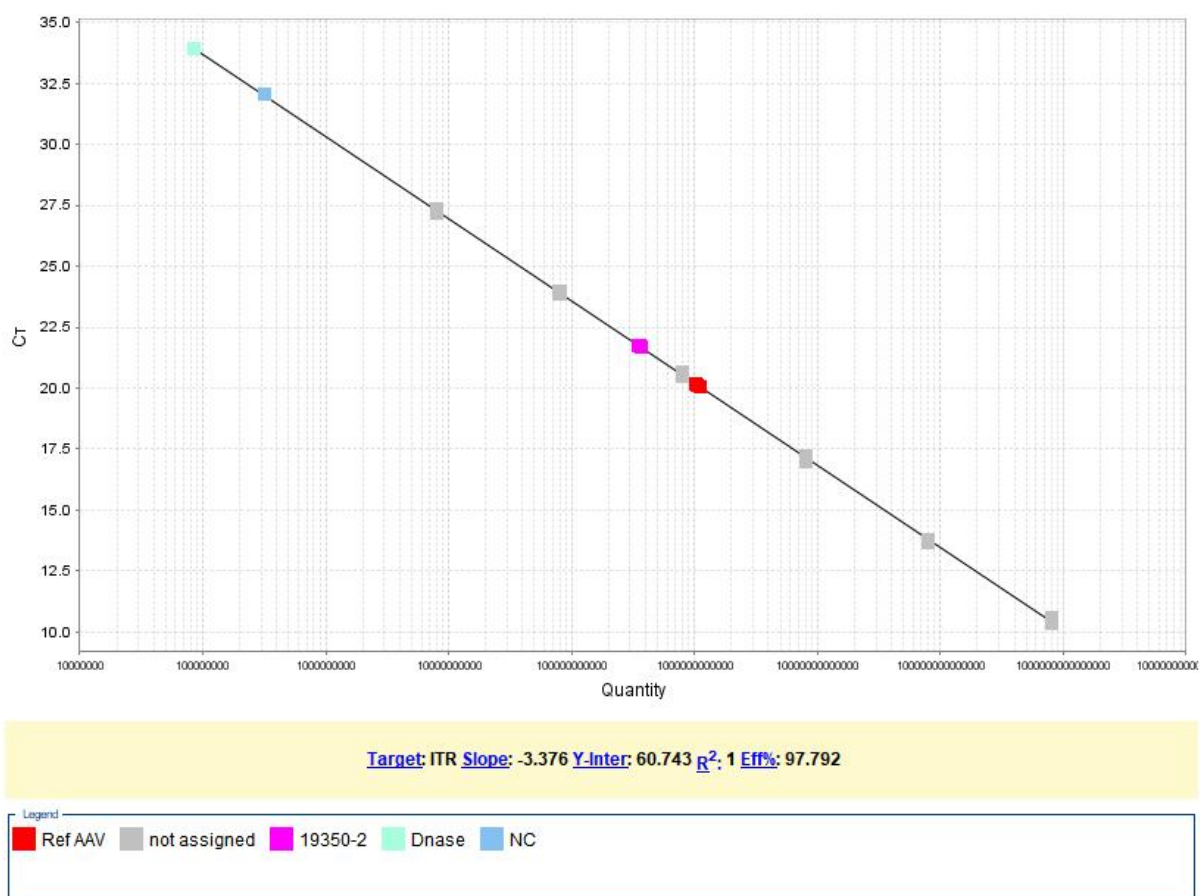


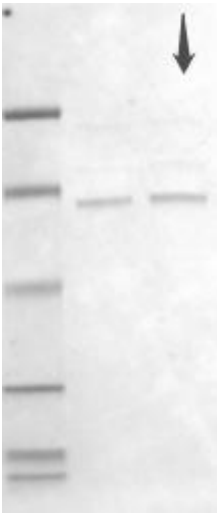
Figure 3 Standard Curve



Item 2: Endotoxin

Name	Sample positive control	Positive control	Neg control	Sample
Result	+	+	—	—

Item 3: Purity (SDS-PAGE)



Maker:97.2KD,66.4KD,44.3KD,29KD,20.1KD,14.3KD

Conclusion: No other significant band

Certificate of Analysis (COA)

Name	ATX001 (Engineered AAV2, contains loop insertion)[CAG-GFP.Barcode 232]	Lot#	19351-2E1
Order#	USJW241008942-22	Date	2024-11-15

Items	Analytical Method	QC results
[Genome Titer]	qPCR	Adjusted Titer: 1.68E+12GC/mL
[Purity]	SDS-PAGE	No other significant band
[Endotoxin]	LAL	<0.2EU/mL

Conclusion:Qualified

* Adjusted Titer: Titers adjusted to order requirements.

Item 1: Genome Titer

Lot#	Quantity Mean GC/ml	Calibration Factor	Dilution factor*	Detected Titer(GC/mL)	Adjusted Titer (GC/mL)
19351-2E1	1.63E+11	0.52	20	1.68E+12	1.68E+12

* Reference AAV:Packgene:AAV2[ssAAV.CAG.EGFP.WPRE.SV40pA],Labeled titer: 5.5E+11(GC/mL)

Figure 1: Sample Location

	1	2	3	4	5	6	7	8	9	10	11	12
A	ITR 8E14	ITR 8E14	ITR 8E14									
B	ITR 8E13	ITR 8E13	ITR 8E13									
C	ITR 8E12	ITR 8E12	ITR 8E12									
D	ITR 8E11	ITR 8E11	ITR 8E11			19351-2 ITR 1.71E11	19351-2 ITR 1.55E11					
E	ITR 8E10	ITR 8E10	ITR 8E10									
F	ITR 8E9	ITR 8E9	ITR 8E9									
G	Ref AAV ITR 1.11E12	Ref AAV ITR 1.03E12	Ref AAV ITR 1.06E12									
H	NC ITR 3.15E8	Dnase ITR 8.55E7										

Figure 2 Amplification curves

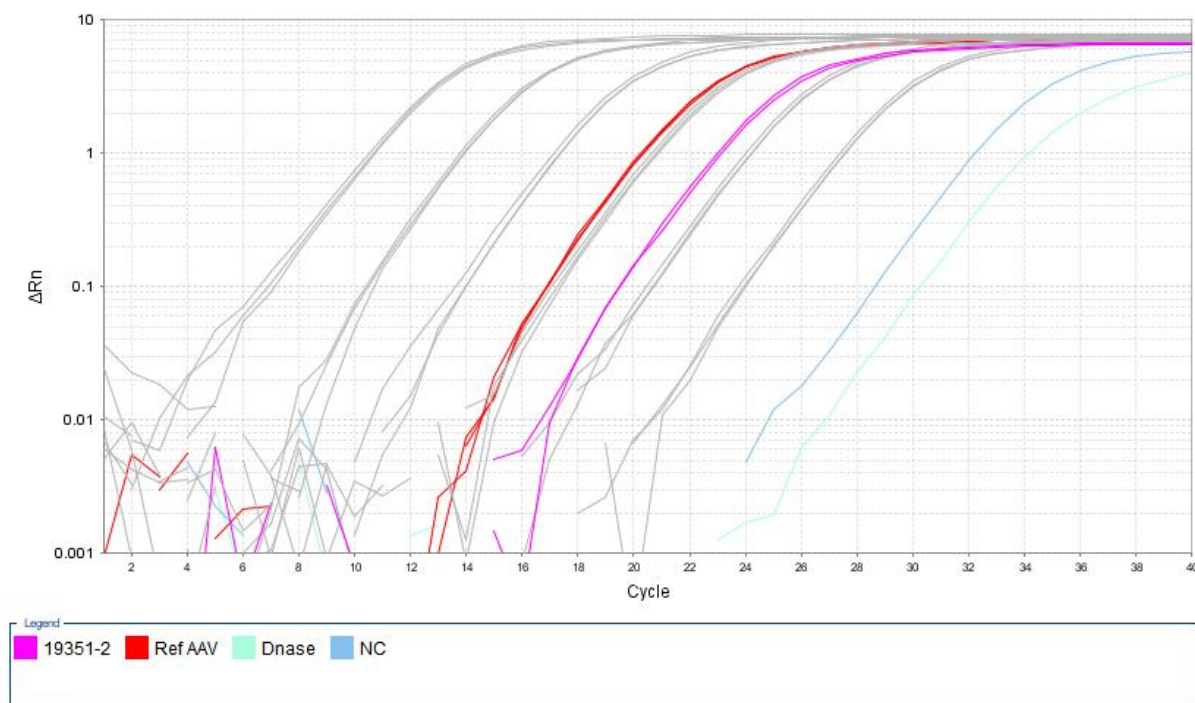
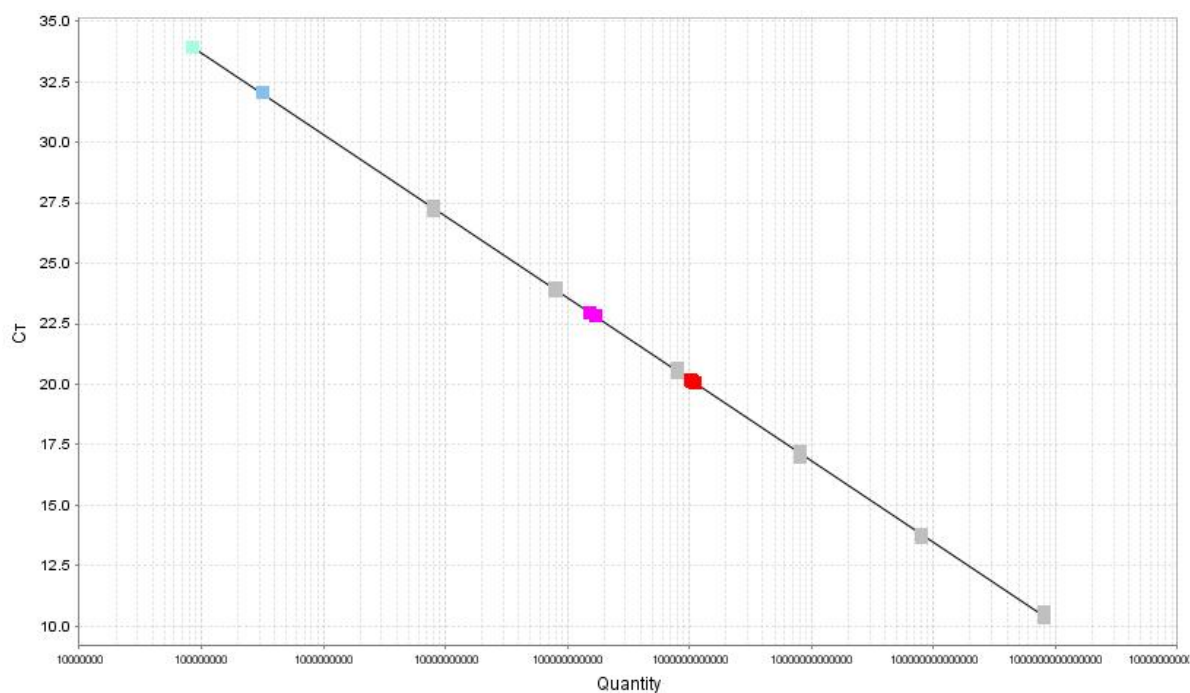


Figure 3 Standard Curve



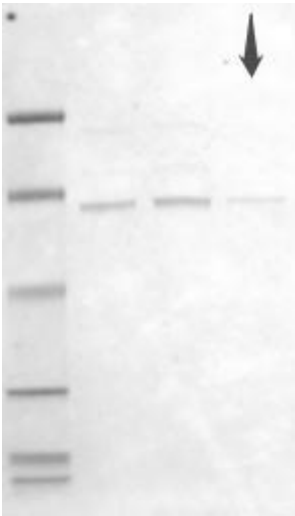
Target: ITR Slope: -3.376 Y-Inter: 60.743 $R^2: 1$ Eff%: 97.792



Item 2: Endotoxin

Name	Sample positive control	Positive control	Neg control	Sample
Result	+	+	—	—

Item 3: Purity (SDS-PAGE)



Maker:97.2KD,66.4KD,44.3KD,29KD,20.1KD,14.3KD

Conclusion: No other significant band

Certificate of Analysis (COA)

Name	ATX001 (Engineered AAV2, contains loop insertion)[CAG-GFP.Barcode 234]	Lot#	19352-2E1
Order#	USJW241008942-24	Date	2024-11-15

Items	Analytical Method	QC results
[Genome Titer]	qPCR	Adjusted Titer: 5.00E+12GC/mL
[Purity]	SDS-PAGE	No other significant band
[Endotoxin]	LAL	<0.2EU/mL

Conclusion:Qualified

* Adjusted Titer: Titers adjusted to order requirements.

Item 1: Genome Titer

Lot#	Quantity Mean GC/ml	Calibration Factor	Dilution factor*	Detected Titer(GC/mL)	Adjusted Titer (GC/mL)
19352-2E1	4.84E+11	0.52	20	5.00E+12	5.00E+12

* Reference AAV:Packgene:AAV2[ssAAV.CAG.EGFP.WPRE.SV40pA],Labeled titer: 5.5E+11(GC/mL)

Figure 1: Sample Location

	1	2	3	4	5	6	7	8	9	10	11	12
A	ITR 8E14	ITR 8E14	ITR 8E14									
B	ITR 8E13	ITR 8E13	ITR 8E13									
C	ITR 8E12	ITR 8E12	ITR 8E12									
D	ITR 8E11	ITR 8E11	ITR 8E11									
E	ITR 8E10	ITR 8E10	ITR 8E10			19352-2 ITR 5E11	19352-2 ITR 4.68E11					
F	ITR 8E9	ITR 8E9	ITR 8E9									
G	Ref AAV ITR 1.11E12	Ref AAV ITR 1.03E12	Ref AAV ITR 1.06E12									
H	NC ITR 3.15E8	Dnase ITR 8.55E7										

Figure 2 Amplification curves

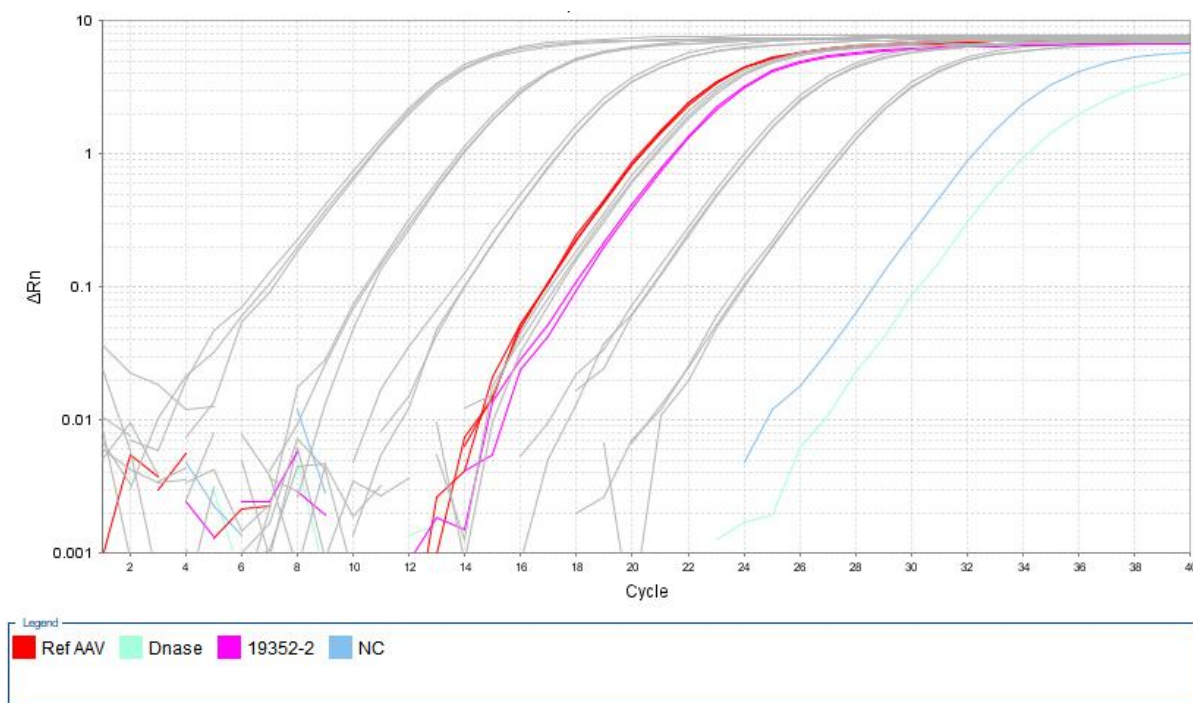
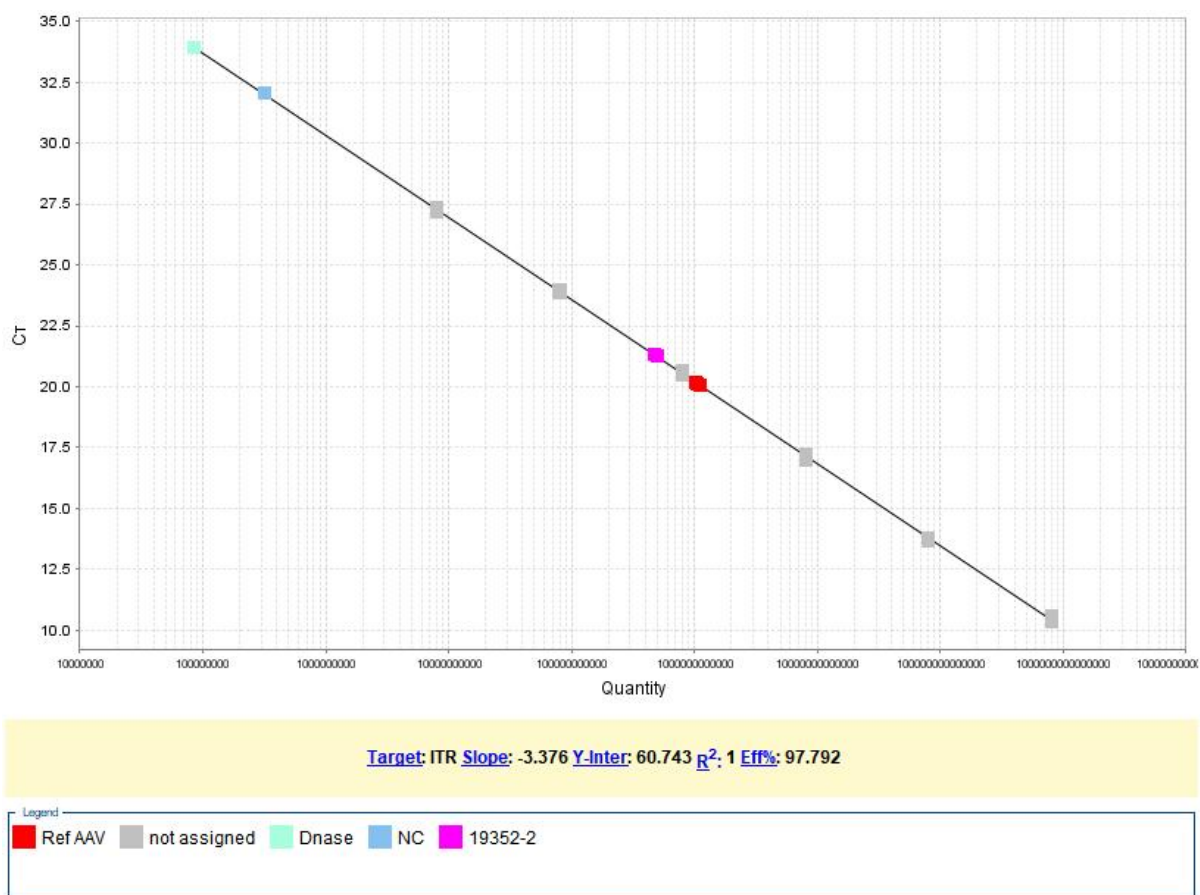


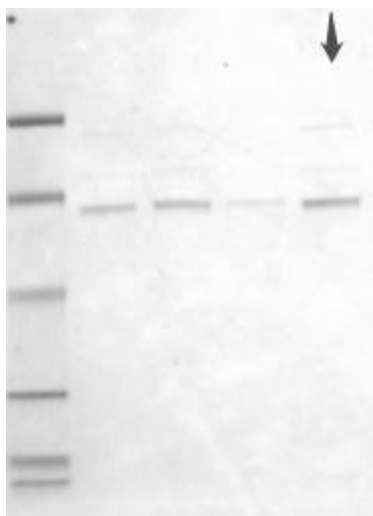
Figure 3 Standard Curve



Item 2: Endotoxin

Name	Sample positive control	Positive control	Neg control	Sample
Result	+	+	—	—

Item 3: Purity (SDS-PAGE)



Maker:97.2KD,66.4KD,44.3KD,29KD,20.1KD,14.3KD

Conclusion: No other significant band

Certificate of Analysis (COA)

Name	ATX001 (Engineered AAV2, contains loop insertion)[CAG-GFP.Barcode 247]	Lot#	19353-2E1
Order#	USJW241008942-26	Date	2024-11-15

Items	Analytical Method	QC results
[Genome Titer]	qPCR	Adjusted Titer: 4.21E+12GC/mL
[Purity]	SDS-PAGE	No other significant band
[Endotoxin]	LAL	<0.2EU/mL

Conclusion:Qualified

* Adjusted Titer: Titers adjusted to order requirements.

Item 1: Genome Titer

Lot#	Quantity Mean GC/ml	Calibration Factor	Dilution factor*	Detected Titer(GC/mL)	Adjusted Titer (GC/mL)
19353-2E1	4.08E+11	0.52	20	4.21E+12	4.21E+12

* Reference AAV:Packgene:AAV2[ssAAV.CAG.EGFP.WPRE.SV40pA],Labeled titer: 5.5E+11(GC/mL)

Figure 1: Sample Location

	1	2	3	4	5	6	7	8	9	10	11	12
A	ITR 8E14	ITR 8E14	ITR 8E14									
B	ITR 8E13	ITR 8E13	ITR 8E13									
C	ITR 8E12	ITR 8E12	ITR 8E12									
D	ITR 8E11	ITR 8E11	ITR 8E11									
E	ITR 8E10	ITR 8E10	ITR 8E10									
F	ITR 8E9	ITR 8E9	ITR 8E9			19353-2 ITR 4.28E11	19353-2 ITR 3.88E11					
G	Ref AAV ITR 1.11E12	Ref AAV ITR 1.03E12	Ref AAV ITR 1.06E12									
H	NC ITR 3.15E8	Dnase ITR 8.55E7										

Figure 2 Amplification curves

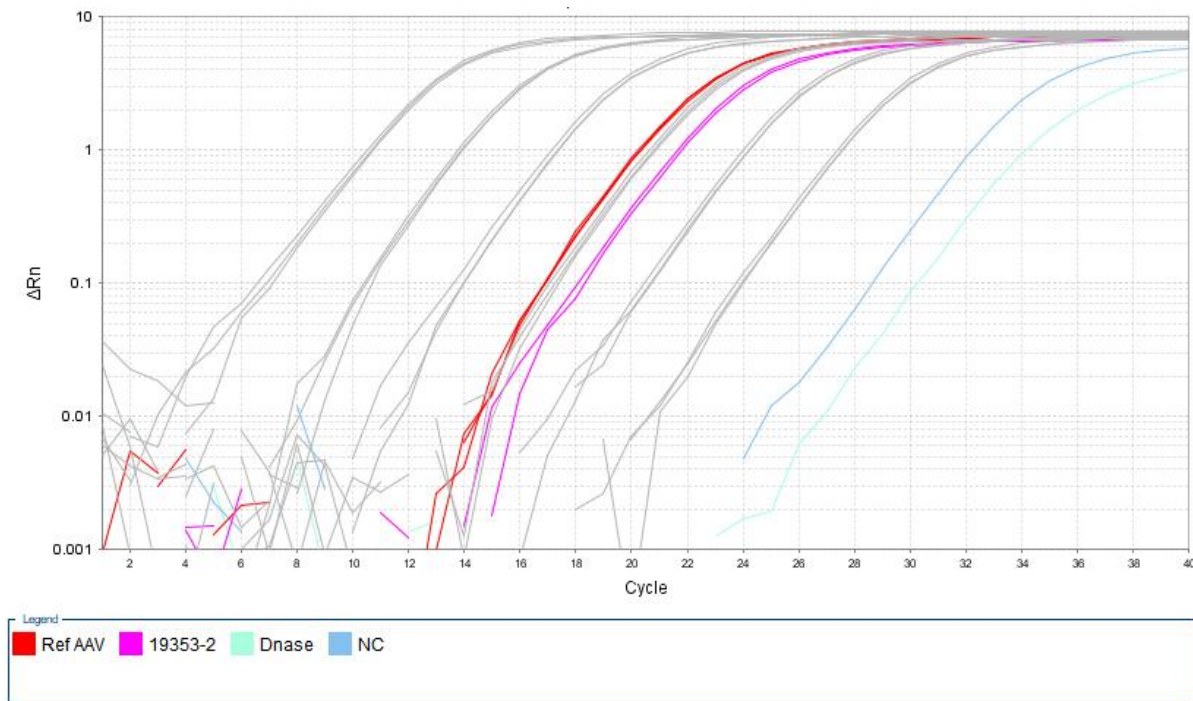
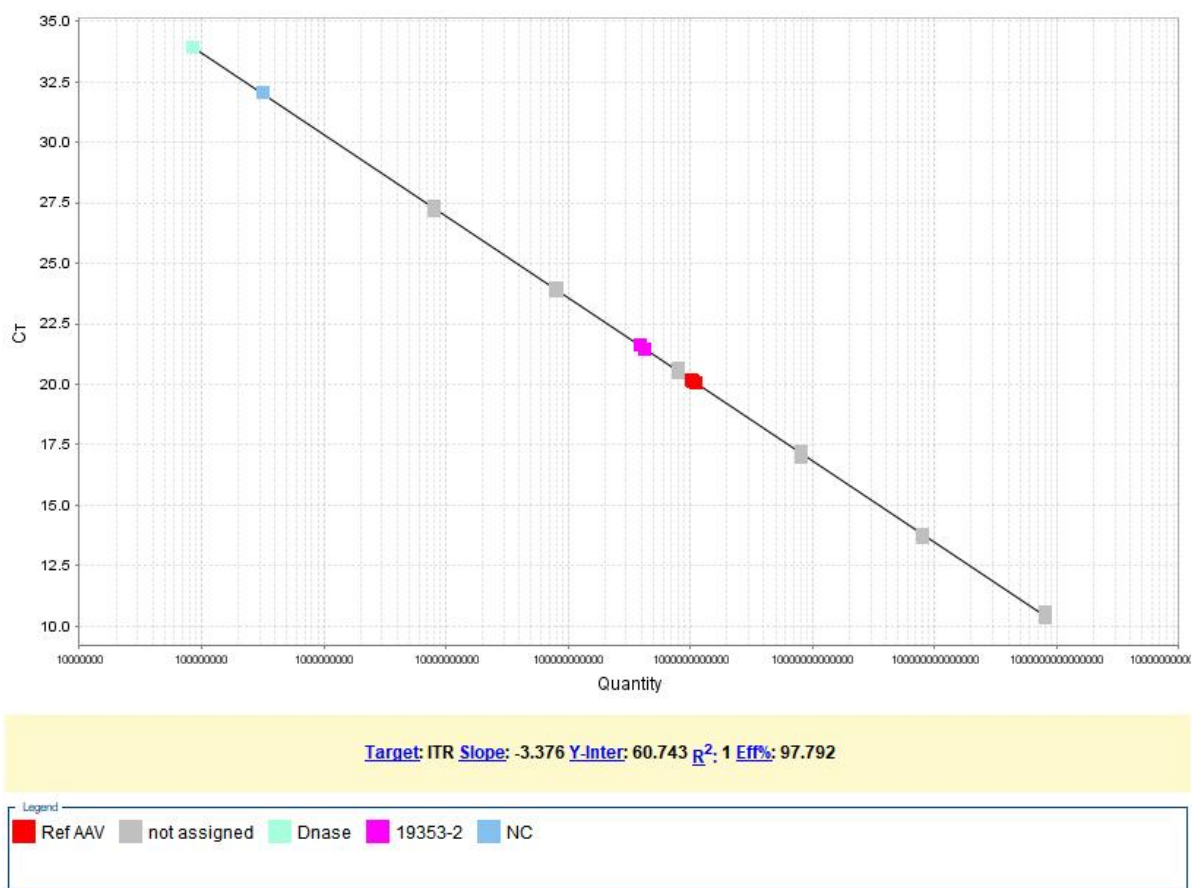


Figure 3 Standard Curve



Item 2: Endotoxin

Name	Sample positive control	Positive control	Neg control	Sample
Result	+	+	—	—

Item 3: Purity (SDS-PAGE)



Maker:97.2KD,66.4KD,44.3KD,29KD,20.1KD,14.3KD

Conclusion: No other significant band