

Cell & Gene Therapy CMC

Write the CMC control section for a cell or gene therapy: the release panel and lot results, starting and ancillary materials, potency, replication-competent virus testing, chain of identity, stability and shipping.

WRITES

3.2.S 3.2.P

3.2.P.5

PRODUCT TYPE

Biologics (ATMPs)

GUIDANCE IT FOLLOWS

FDA Chemistry, Manufacturing, and Control (CMC) Information for Human Gene Therapy Investigational New Drug Applications (2020); FDA Testing of Retroviral Vector-Based Human Gene Therapy Products for Replication Competent Retrovirus During Product Manufacture and Patient Follow-up (2020); FDA Potency Assurance for Cellular and Gene Therapy Products (2023 draft); FDA Considerations for the Development of CAR T Cell Products (2024); 21 CFR 1271; 21 CFR 610.12; EU GMP Part IV (ATMPs);

TIME TO LEARN

About 20 minutes with the example

EMA Guideline on quality, non-clinical and clinical requirements for investigational ATMPs (EMA/CHMP/BWP/QWP/EMA Guideline on quality, non-clinical and clinical aspects of medicinal products containing genetically modified cells (EMA/CAT/GTWP/67163 Rev. 1); Ph. Eur. 5.14, 2.6.27, 2.6.1, 2.6.7; Directive 2004/23/EC; Directive 2001/18/EC

Open the tool at <https://app.cmcfy.com/app/cell-gene-therapy.html>. This guide is also online at <https://cmcfy.com/guides/cell-gene-therapy.html>

1. What this module does

You choose the product type (AAV vector, lentiviral vector, autologous gene-modified cells such as CAR-T, allogeneic cells or unmodified autologous cells) and the stage, then enter the release tests with their criteria and the result of each lot.

The tool checks every result against its criterion, the tests expected for the product type, vector copy number, viability, phase-appropriate potency, donor or patient material, chain of identity, the vector used as starting material, ancillary materials, stability, shipping and (for the EU) the GMO environmental risk assessment.

What you get

- A Word or PDF document: Control of the product (specification, batch results, control strategy, starting materials, stability).

2. Before you start

Have these ready. You can still start with the example data and replace it piece by piece.

- The release specification and results for each lot.
- Donor or patient material controls, vector lot and ancillary materials.
- Potency assay description, stability and shipping data.

Your data stays on your computer. The bar under the title counts every network request the page makes; it stays at 0 because your data never leaves your computer. [Download offline](#) [copy](#) saves a single file that opens from your own computer with all network access blocked, for air-gapped PCs.

3. Step by step with the example

Open the tool at app.cmcfy.com/app/cell-gene-therapy.html. The example is loaded when the page opens, so every panel already has realistic data. Follow the steps to see how it works, then replace the example with your own data.

1

Describe the product

Choose the regions, product type and stage, and list the lots.

Cell & Gene Therapy CMC

[Projects](#)
[All modules](#)
[Training](#)
[Security](#)

Runs entirely on this device

Training guide

PROJECTS

Keep this work in a project, with version history and your company logo on every report.

Choose projects folder

Keep in this browser

Network requests since load: 0

Counted live by your browser. Your data never leaves this device.

Works offline

Everything the tool needs is inside this page.

Locked offline copy

Save a single file that opens from your own computer with all network access blocked.

3.2.S

3.2.P

3.2.P.5

Save project

Open project

Start blank

Load example

Write the CMC control section for a cell or gene therapy (ATMP): the release panel and lot results, starting materials, replication-competent virus testing, potency and stability. The tool checks the tests expected for the product type and phase, every lot result against its criterion, and the controls specific to autologous and allogeneic products.

Guidance: FDA Chemistry, Manufacturing, and Control (CMC) Information for Human Gene Therapy Investigational New Drug Applications (2020); FDA Testing of Retroviral Vector-Based Human Gene Therapy Products for Replication Competent Retrovirus During Product Manufacture and Patient Follow-up (2020); FDA Potency Assurance for Cellular and Gene Therapy Products (2023 draft); FDA Considerations for the Development of CAR T Cell Products (2024); 21 CFR 1271; 21 CFR 610.12 / Ph. Eur. 2.6.1; EU GMP Part IV (ATMPs); EMA Guideline on quality, non-clinical and clinical requirements for investigational ATMPs (EMA/CHMP/BWP/QWP/IWP/123/2019); EMA Guideline on quality, non-clinical and clinical aspects of medicinal products containing genetically modified cells (EMA/CAT/GTWP/671639/2008 Rev. 1); Ph. Eur. 5.14, 2.6.27, 2.6.1, 2.6.7; Directive 2004/23/EC; Directive 2001/18/EC

1 · PRODUCT

Product

CMX-CAR19 (autologous anti-CD19 CAR-T cells), suspension

Regulations

Both US and EU

Product type

Autologous gene-modified cells

Stage

Phase 2

Lots reported (";" separated)

CAR19-001;CAR19-002;CAF

RESULT

3 lots · all release results within criteria · release panel complete

Autologous gene-modified cells (for example CAR-T), phase 2. VCN 1.8, 2.1, 2.4 copies/cell. Viability 88, 91, 85%. Potency: Potency (IFN-γ release on CD19+ targets; pg/mL).

PRODUCT TYPE	RELEASE TESTS	LOTS
Autologous gene-modified cells	12	3

STAGE

2 Enter release tests and controls

One row per test with the results per lot separated by ";" in lot order. Then describe the starting materials, potency, stability and shipping.

3 Read the result

The headline sums up the lots, the results and the panel: "3 lots · all release results within criteria · release panel complete".

CMCfy training guide · Cell & Gene Therapy CMC

Page 4 of 10

RELEASE RESULTS BY LOT						
TEST	METHOD	CRITERION	CAR19-001	CAR19-002	CAR19-003	C
Identity (CAR expression)	Flow cytometry	CAR+ detected	CAR+ detected	CAR+ detected	CAR+ detected	p
Viability (%)	Flow cytometry (7-AAD)	≥ 70	88	91	85	p
Viable CAR+ T cells (×10 ⁸ per bag)	Flow cytometry	1.0–2.0	1.4	1.6	1.2	p
Transduction efficiency (% CAR+)	Flow cytometry	≥ 15	42	38	47	p
Vector copy number (copies/transduced cell)	ddPCR	≤ 5	1.8	2.1	2.4	p
Potency (IFN-γ release on CD19+ targets; pg/mL)	Co-culture ELISA	≥ 500	2150	1890	2430	p
Purity (% CD3+)	Flow cytometry	≥ 80	96	94	97	p
Replication-competent lentivirus	qPCR (VSV-G) on end-of-production cells	Not detected	Not detected	Not detected	Not detected	p
Residual CD3/CD28 beads (per 3×10 ⁶ cells)	Microscopy	≤ 100	12	8	15	p
Sterility	Ph. Eur. 2.6.27 / 21 CFR 610.12	No growth	No growth	No growth	No growth	p
Endotoxin (EU/mL)	LAL kinetic chromogenic	≤ 5.0	<0.5	<0.5	<0.5	p
Mycoplasma	qPCR (NAT)	Not detected	Not detected	Not detected	Not detected	p

Release results by lot.

4

Resolve alerts and download

A failing result and an uncontrolled chain of identity are critical. Missing expected tests, VCN above 5, viability below 70%, potency without a criterion at a late stage and missing controls are flagged. Then preview and download.

Preview and review before download

Close

Review

Checked on this device: gaps in the data, gaps in the report against ICH and regional expectations, and differences from the protocol.

No open gaps

No open gaps: the data, the report and the protocol (if given) match every rule checked, or you accepted the rest. Read the preview before you sign.

Compare with the protocol

Load or paste the approved protocol. Every requirement and acceptance criterion in it is looked for in the report.

Paste the protocol text here, or load the file.

Load protocol file Compare

AI review against guidance

AI reads the report, the protocol and the data against the ICH, FDA or EMA requirements for these sections, and lists gaps with the guideline it applies, protocol and data mismatches, and how to fix them. You see exactly what is sent before anything leaves this device. Results and pass/fail are not changed.

Run AI review

3.2.P.5 Control of the product: release testing and batch results

CMX-CAR19 (autologous anti-CD19 CAR-T cells), suspension for infusion · Autologous gene-modified cells (for example CAR-T) · US (FDA) and EU (EMA)

Product	CMX-CAR19 (autologous anti-CD19 CAR-T cells), suspension for infusion
Product type	Autologous gene-modified cells (for example CAR-T)
Stage	Phase 2
Lots	CAR19-001, CAR19-002, CAR19-003
Regulations	US (FDA) and EU (EMA)

1. Specification

Test	Method	Acceptance criterion
Identity (CAR expression)	Flow cytometry	CAR+ detected
Viability (%)	Flow cytometry (7-AAD)	≥ 70
Viable CAR+ T cells (×10 ⁶ per bag)	Flow cytometry	1.0–2.0
Transduction efficiency (% CAR+)	Flow cytometry	≥ 15
Vector copy number (copies/transduced cell)	ddPCR	≤ 5
Potency (IFN-γ release on CD19+ targets; pg/mL)	Co-culture ELISA	≥ 500
Purity (% CD3+)	Flow cytometry	≥ 80
Replication-competent lentivirus	qPCR (VSV-G) on end-of-production cells	Not detected
Residual CD3/CD28 beads (per 3×10 ⁶ cells)	Microscopy	≤ 100
Sterility	Ph. Eur. 2.6.27 / 21 CFR 610.12	No growth
Endotoxin (EU/mL)	LAL kinetic chromogenic	≤ 5.0
Mycoplasma	qPCR (NAT)	Not detected

Download Word (.docx) Download PDF

4. Field reference

Every input in the tool, panel by panel. Tables accept rows pasted straight from Excel, comma-separated text, or a loaded file; check the columns after loading a file. Fields marked “when it applies” appear only for the choices that need them.

1 • Product

FIELD	WHAT TO ENTER
Product	Type it in.
Regulations	Choose one: US: FDA (IND or BLA); EU: EMA (IMPD or MAA); Both US and EU.
Product type	Choose one: AAV gene therapy vector; Lentiviral or retroviral vector; Autologous gene-modified cells (for example CAR-T); Allogeneic cell therapy; Autologous cell therapy (not gene-modified).
Stage	Choose one: Phase 1; Phase 2; Phase 3 (pivotal); Marketing application.
Lots reported (";" separated)	Lot names in the same order as the results. Type it in.

2 · Release testing

One row per test. Criterion: a range, $\geq$ or $\leq$ limit, a statement such as Negative, or "Report result". Results per lot separated by ";", in the order of the lots.

FIELD	WHAT TO ENTER
Release tests and results	Write a criterion as a range, a $\geq$ or $\leq$ limit, Not detected, or Report result. A table. Columns: Test Method Acceptance criterion Results per lot. Paste rows from Excel, type comma-separated rows, or press Load file (Excel, CSV, Word, PDF or a scan).

3 · Starting materials and controls

FIELD	WHAT TO ENTER
Donor or patient material when it applies	Type or paste text.
Chain of identity and chain of custody controlled from collection to infusion when it applies	Tick the box if it applies.
Viral vector used as starting material and its release when it applies	Type or paste text.
Ancillary materials (USP <1043>, Ph. Eur. 5.2.12)	Type or paste text.
Potency assay and link to mechanism of action	Type or paste text.
Stability (shelf life, post-thaw and in-use)	Type or paste text.
Shipping and storage at the clinical site	Type or paste text.
GMO environmental risk assessment done when it applies	Tick the box if it applies.

5. Checks and alerts

Calculations and pass/fail outcomes always come from fixed rules, never from AI. The tool checks:

- Each lot result against its criterion.
- Expected tests for the product type (for example rcAAV and full/empty capsids for AAV; RCL, VCN and transduction for CAR-T).
- $VCN \leq 5$ copies per cell, viability $\geq 70\%$, quantitative potency before pivotal studies.
- Donor or patient material, chain of identity, vector, ancillary materials, stability, shipping, GMO assessment (EU).

How to resolve alerts

- **Critical** alerts block the download and must be fixed in the data. A failing result can instead be linked to an investigation: type the investigation reference and outcome and press **Record**.
- **Major** alerts block the download until you fix them or type a justification and press **Accept justification**.
- **Minor** alerts do not block anything. They are listed in the documents so the reviewer sees them.
- Some alerts have an **Apply fix** button that makes the change for you. **Reopen** undoes a justification.

Narratives

This module writes: Control strategy summary. Each narrative is written by fixed rules from your data. You can edit it in the box. **Rewrite with AI** (where your plan includes AI) rewrites it in regulatory style; every number in the text is checked against your data, and a number that does not match is flagged under the box and blocks the download until you correct it. **Reset to rule-based text** brings back the original.

6. Preview, gap review and download

1. Press **Preview** next to a section, or **Preview and download all**. The document opens in a preview window, exactly as the Word file will read.
2. The left side lists the review, run on your device: **gaps in the data** (missing values, open alerts), **gaps in the report** (missing content the guidance expects, placeholders such as [site] or TBD left in the text) and, if you load the approved protocol under **Compare with the protocol**, **differences from the protocol** (requirements, criteria, batches or time points it plans that the report does not show).
3. Click a finding to jump to the place in the preview. Fix it in the tool, then preview again.

4. Optional **AI review**: a second read for contradictions, claims without data and vague wording. The report text is sent to Claude only when you press it, and only where your plan includes AI. Check every AI finding; AI can be wrong.
5. Press **Download Word (.docx)** or **Download PDF**. With several documents open, **All as Word (.zip)** and **All as PDF (.zip)** download the set. The download buttons stay disabled while a blocking alert is open; the preview still works.

Documents this module produces

SECTION	DOCUMENT
3.2.P.5	Specification, batch results and control strategy

Use **Save project** to keep your work as a file on your computer, and **Open project** to continue later. **Start blank** clears every field; **Load example** brings the example back. Nothing is stored on CMCfy servers.

7. US and EU submissions

US follows the FDA gene therapy CMC guidance (2020), the RCR testing guidance (2020), the potency assurance draft guidance (2023), the CAR T guidance (2024) and 21 CFR 1271. EU follows EU GMP Part IV, the EMA guidelines for investigational ATMPs and genetically modified cells, Ph. Eur. 5.14, and the GMO directives.

8. Working with other modules

- Viral safety of cell substrates is in the Viral Safety Studio module.
- Stability data can be evaluated in the Biologics Stability Studio module.

9. Common pitfalls

- Using a potency assay that does not reflect the mechanism of action.
- Testing RCL only on the vector and not on the end-of-production cells.

10. Practice exercises

Each exercise starts from the example (press **Load example** to reset). We run these on every update of this guide, so the result you see should match.

1. **Enter a high vector copy number**

Change the VCN results to 1.8;2.1;6.2.

EXPECTED RESULT

The headline shows one test failed.

2. **Switch to an AAV product**

Set the product type to AAV gene therapy vector.

EXPECTED RESULT

The headline shows expected tests missing from the panel.

Guide updated 28 October 2026 for the current version of the tool. CMCfy drafts are for review by a qualified person before use in a submission.