

FIG. 1

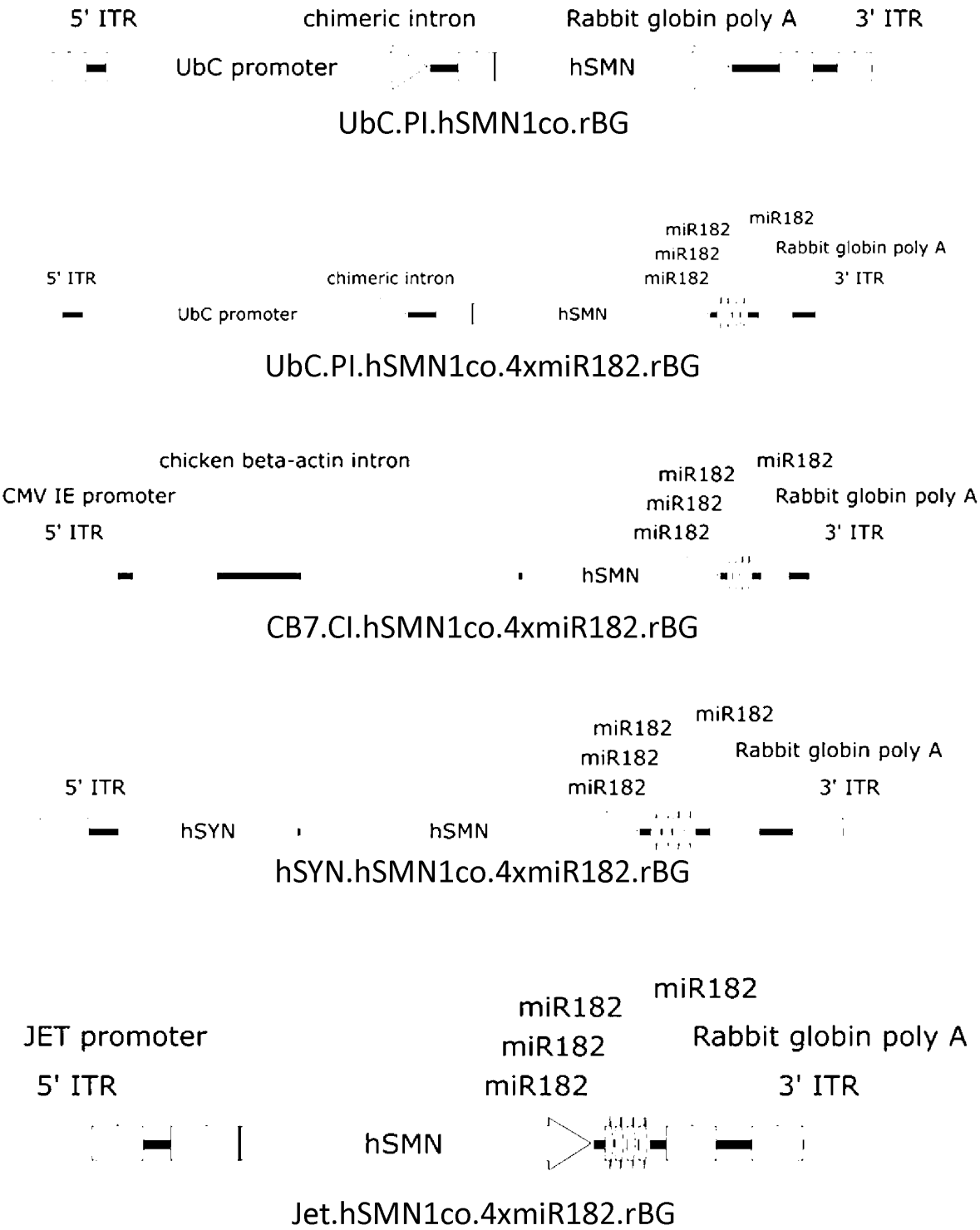


FIG. 2

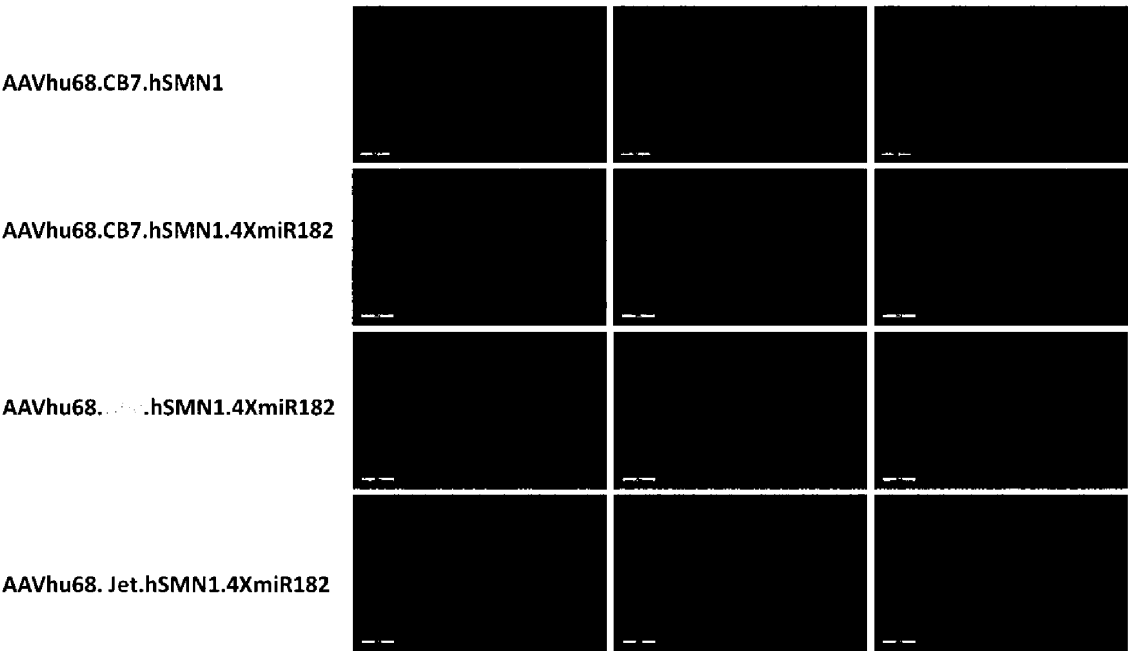


FIG. 3

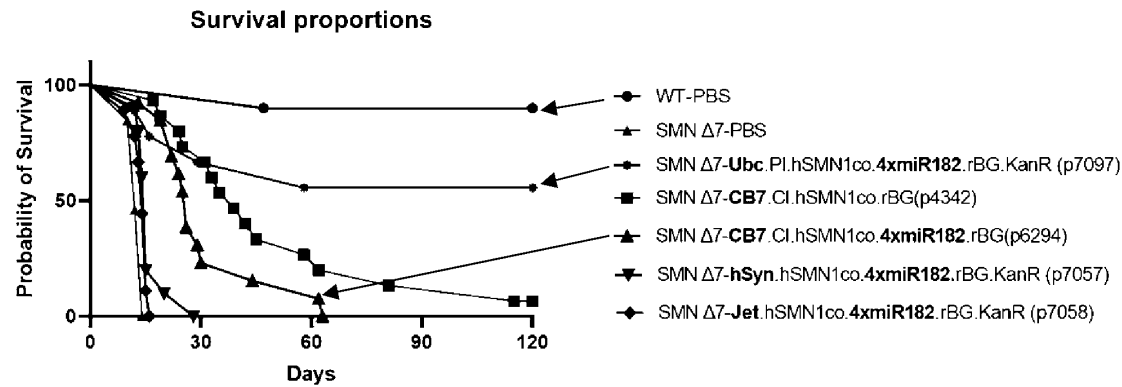


FIG. 4

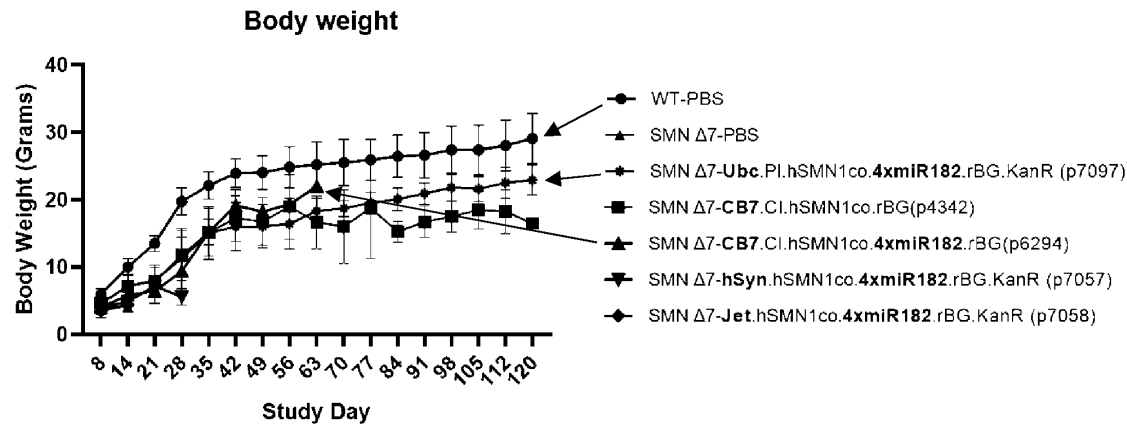


FIG. 5

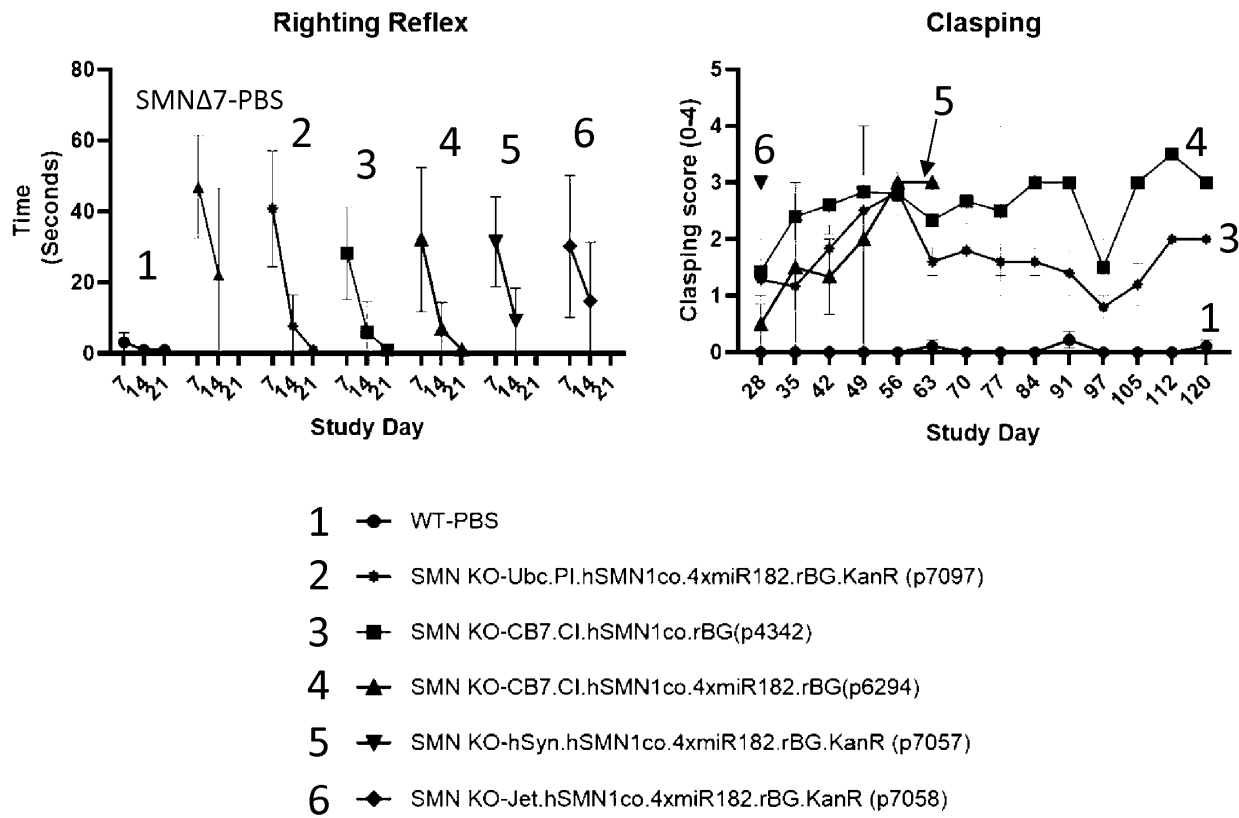


FIG. 6

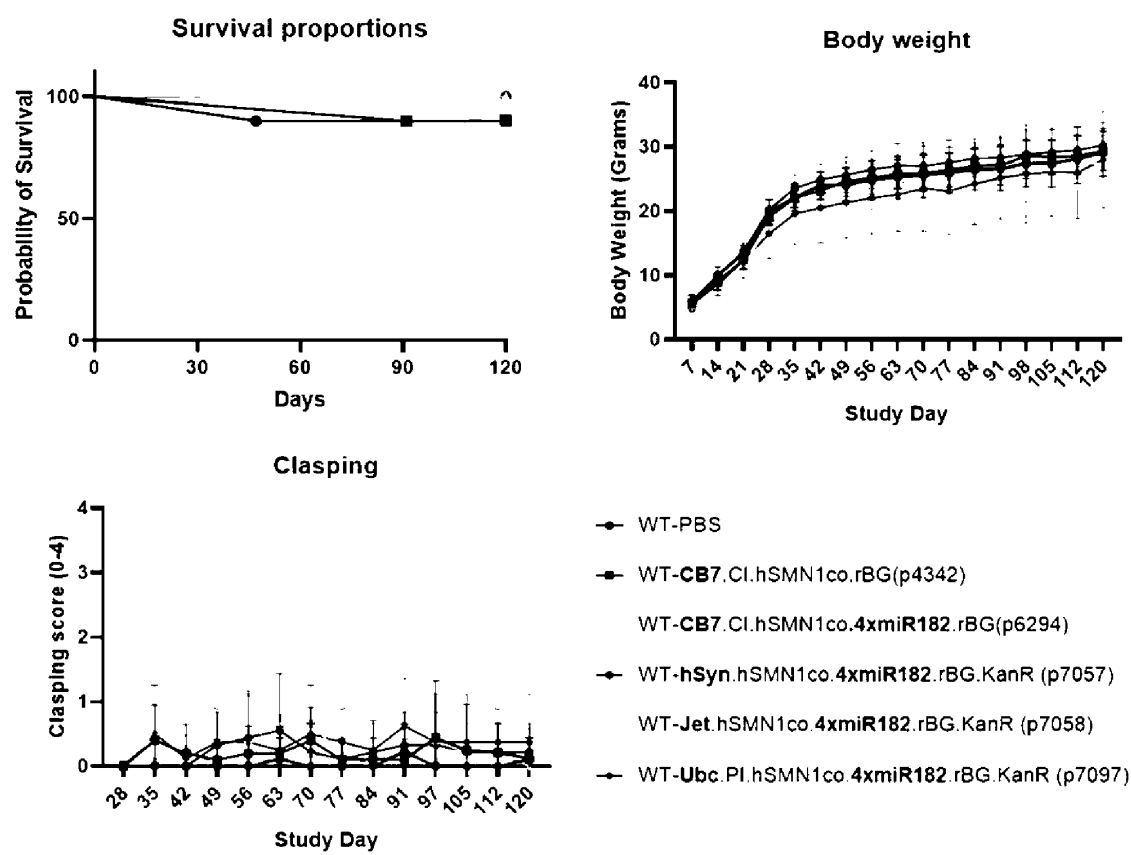


FIG. 7A

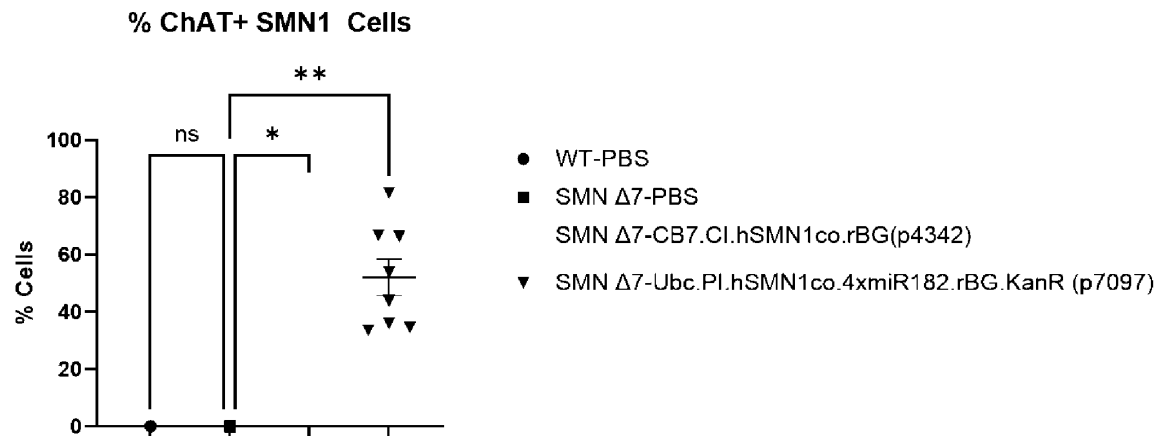


FIG. 7B

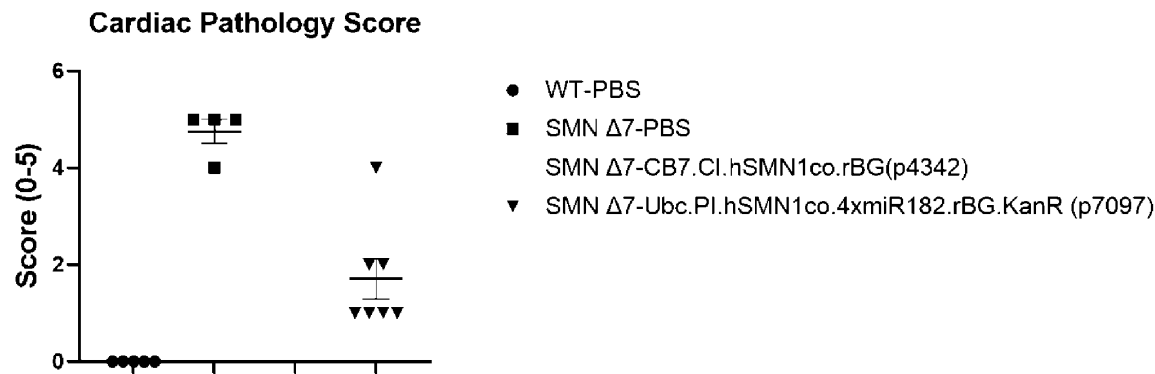


FIG. 8A
SMN ISH Intensity

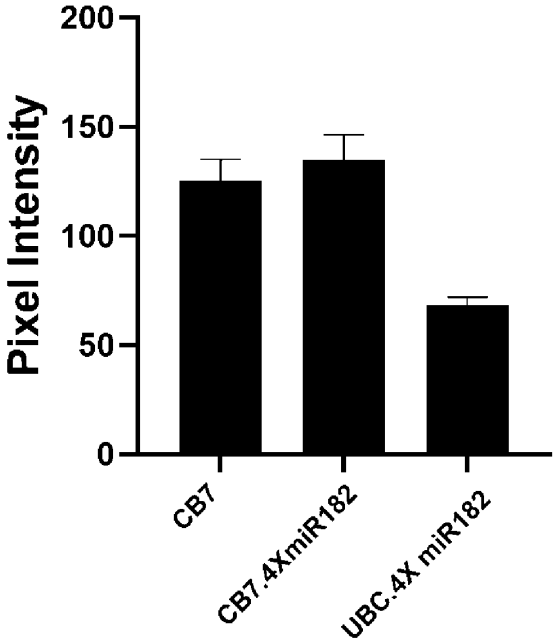


FIG. 8B
ISH analysis

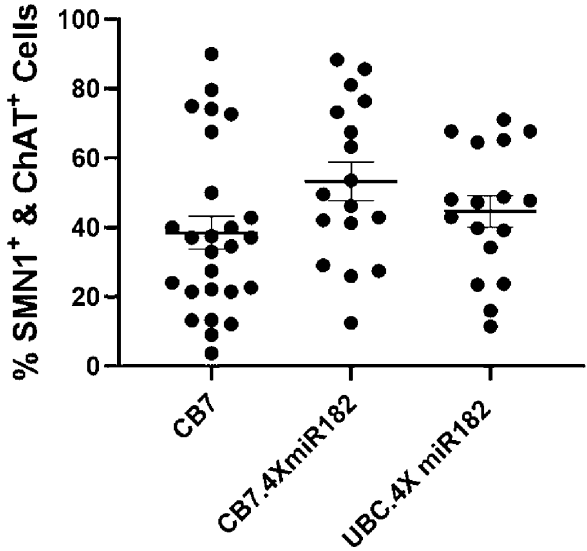


FIG. 9

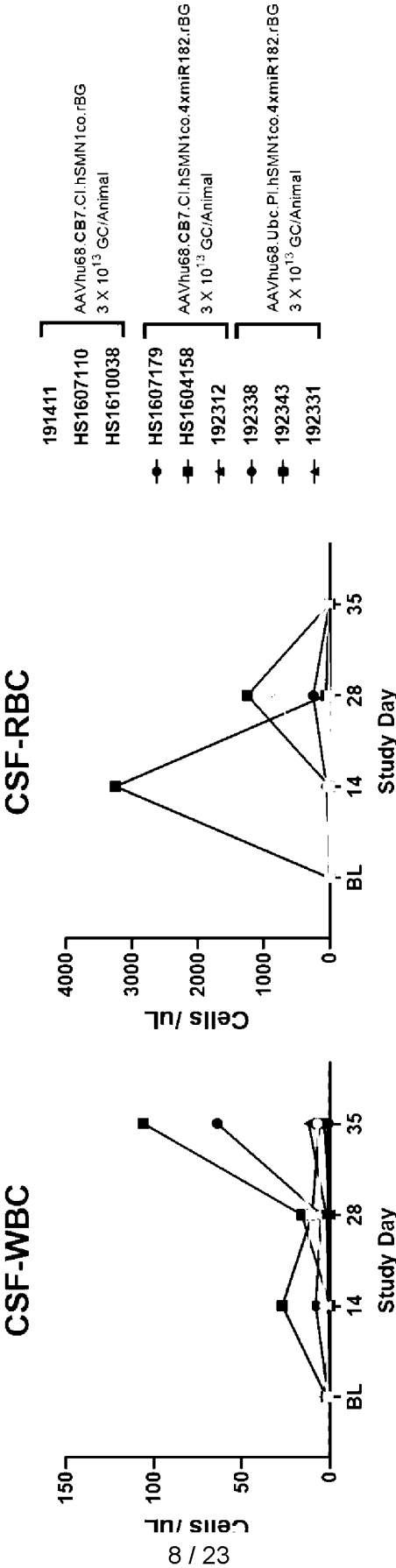


FIG. 10

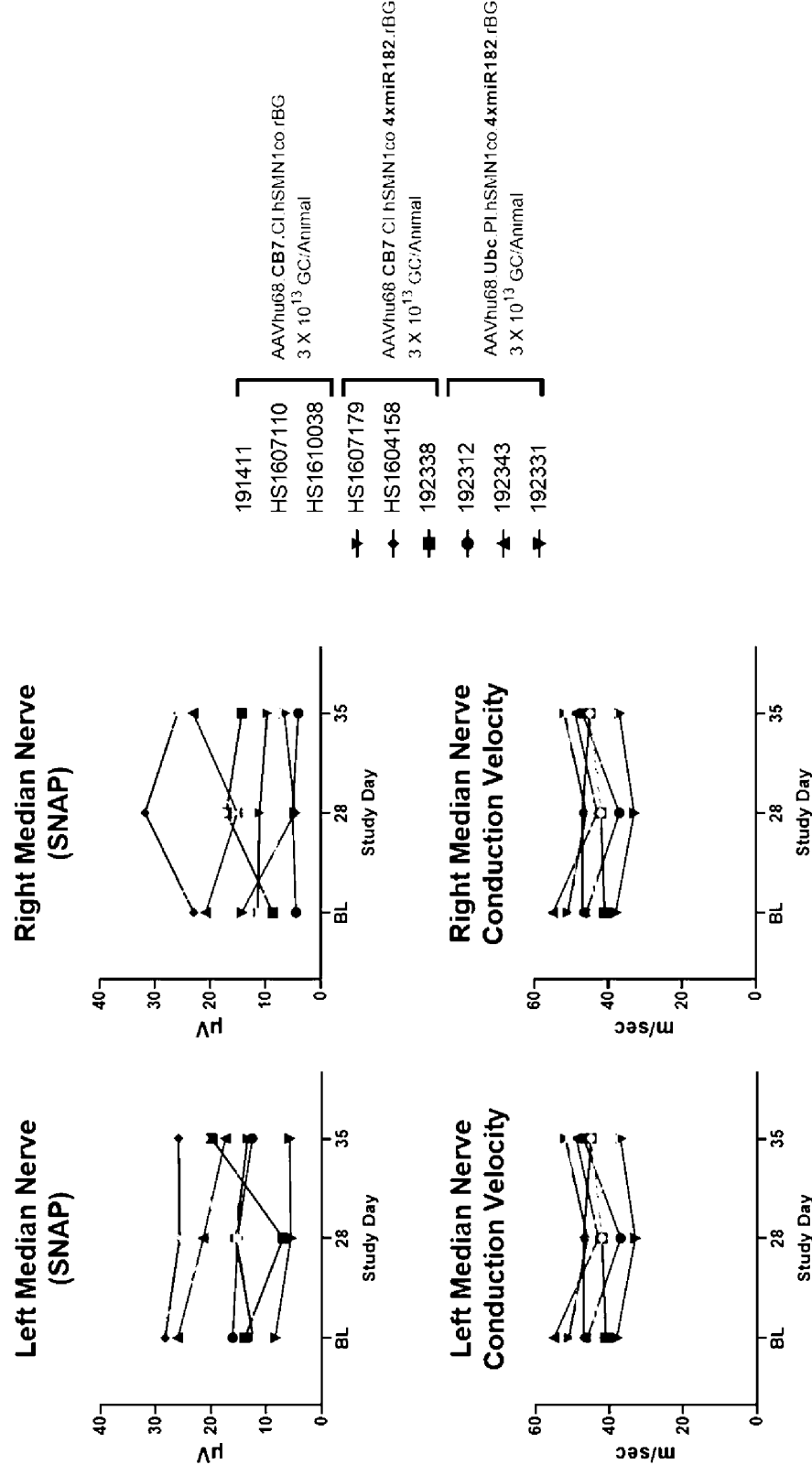


FIG. 11

DRG: Neuronal Degeneration

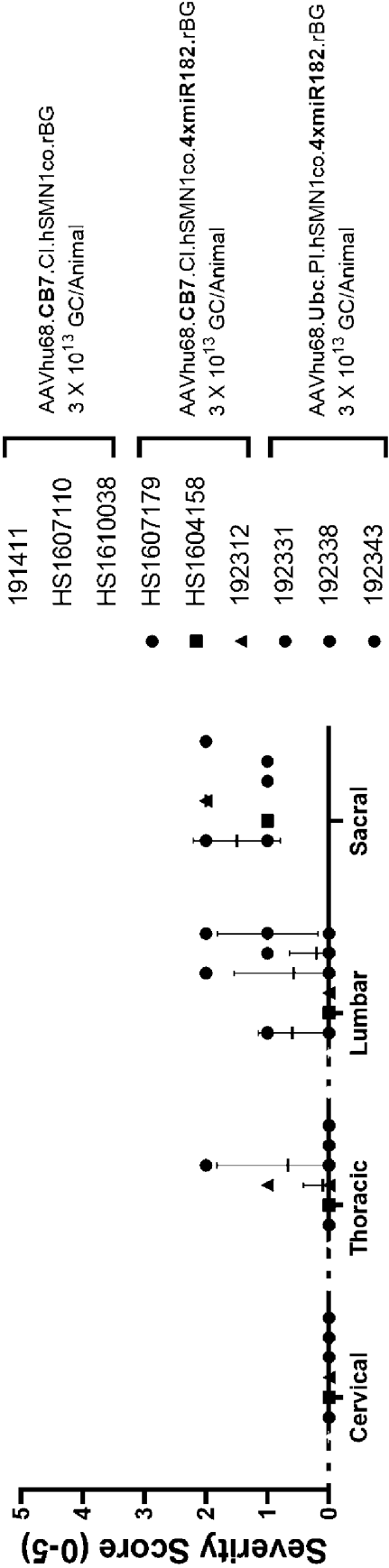


FIG. 12

SC: Axonopathy Scores

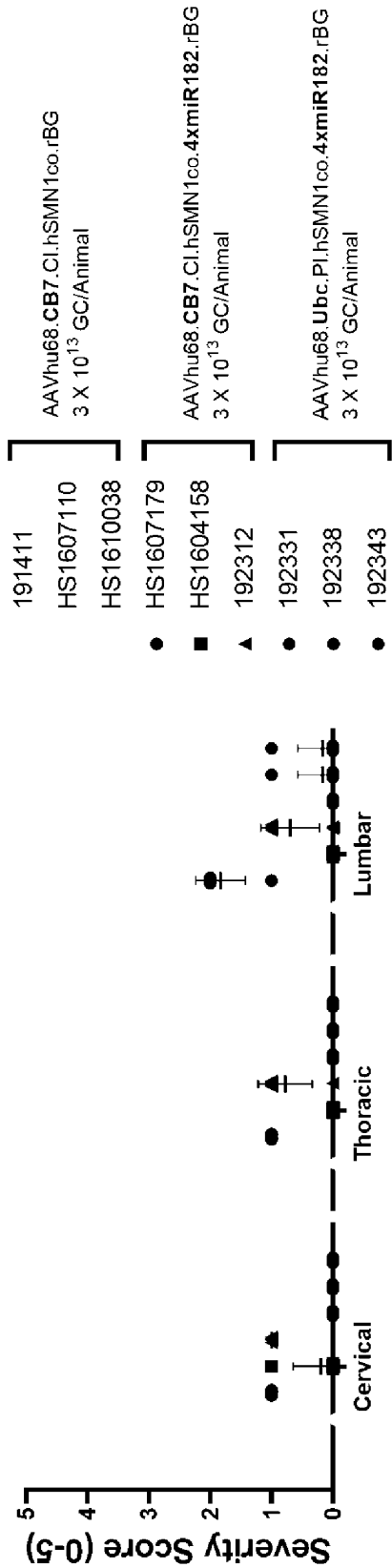


FIG. 13A

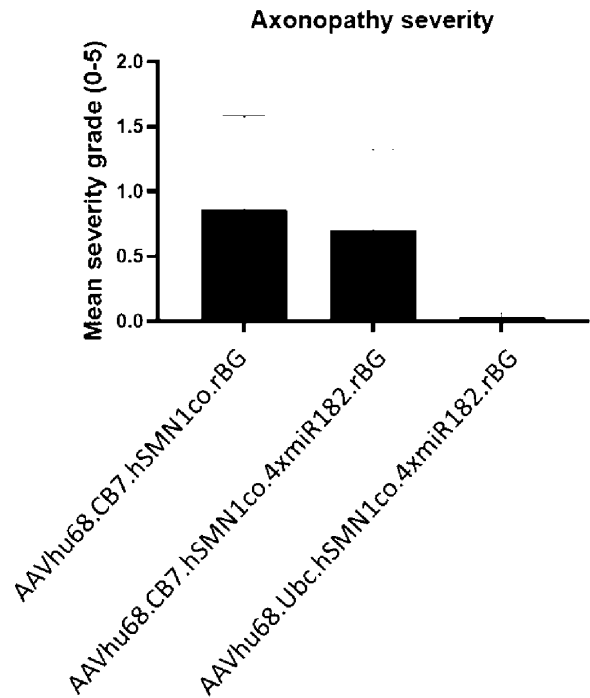


FIG. 13B

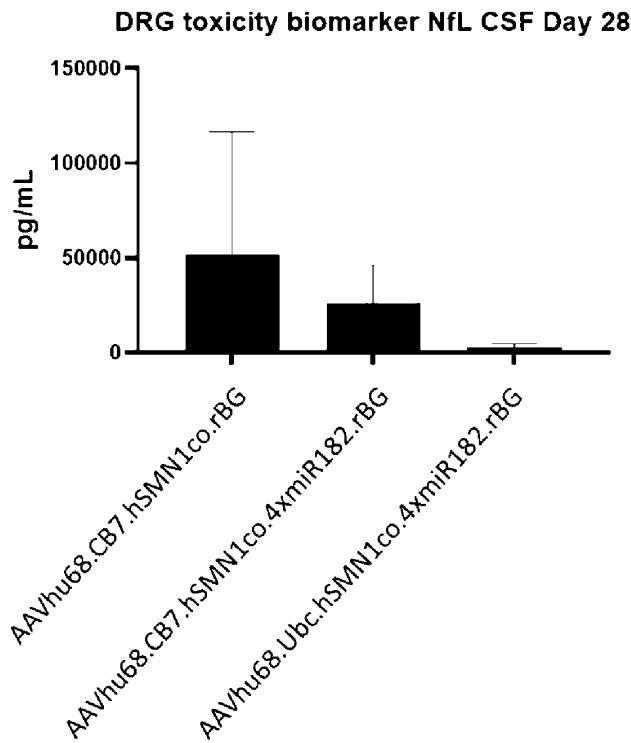


FIG. 14A

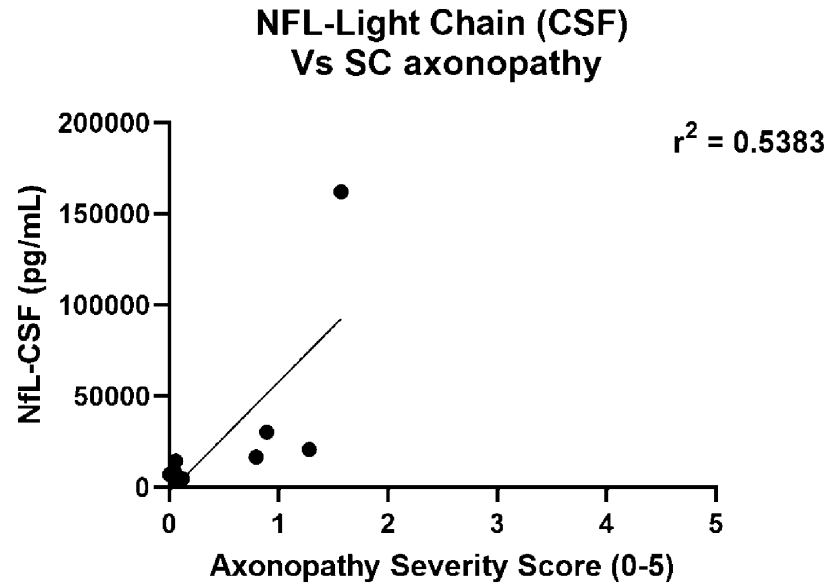
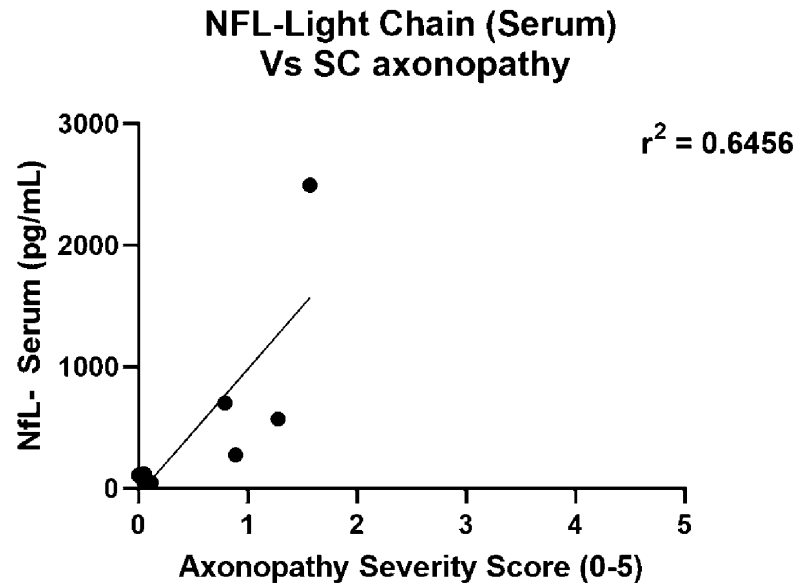


FIG. 14B



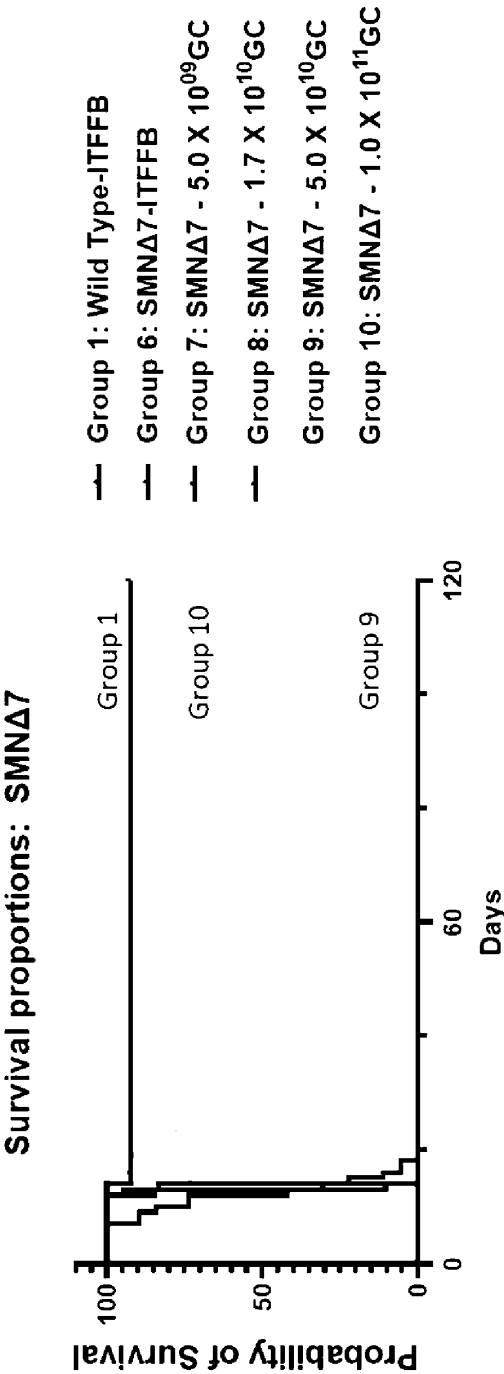


FIG. 15

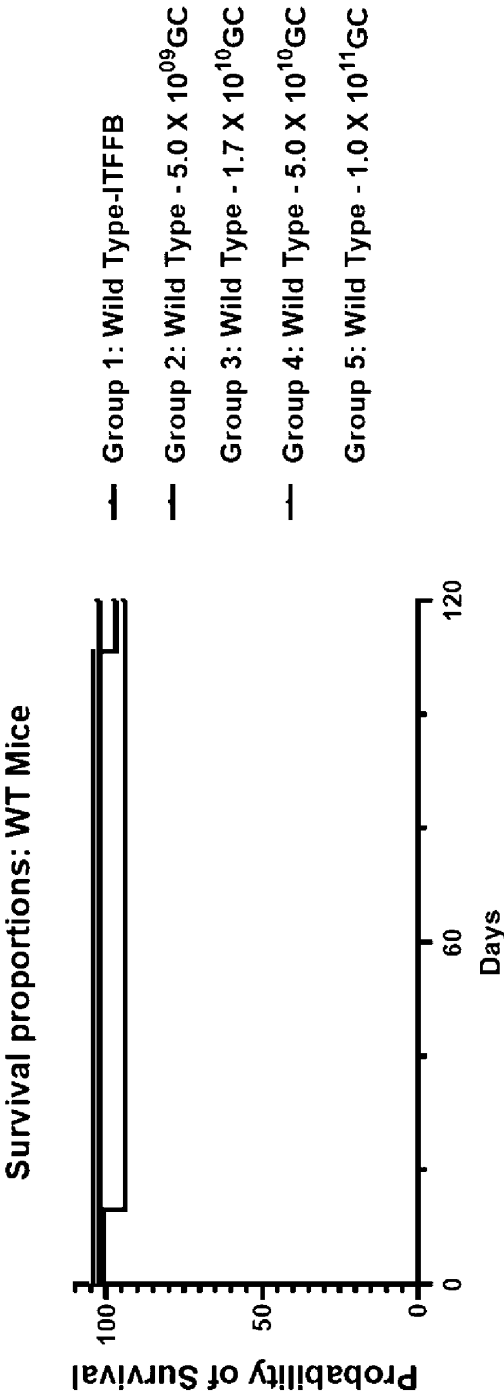


FIG. 16

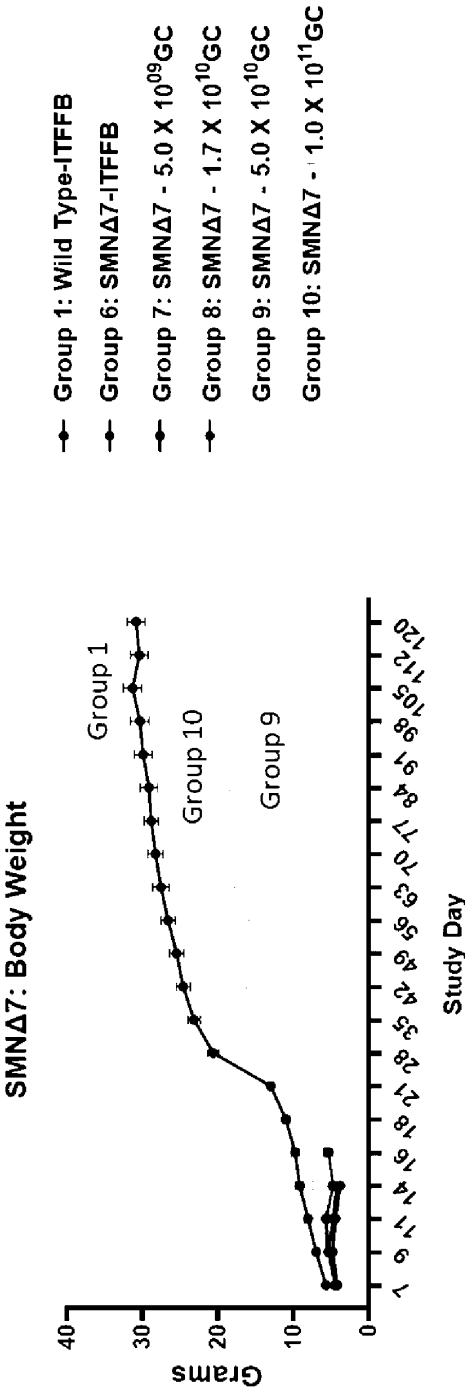


FIG. 17

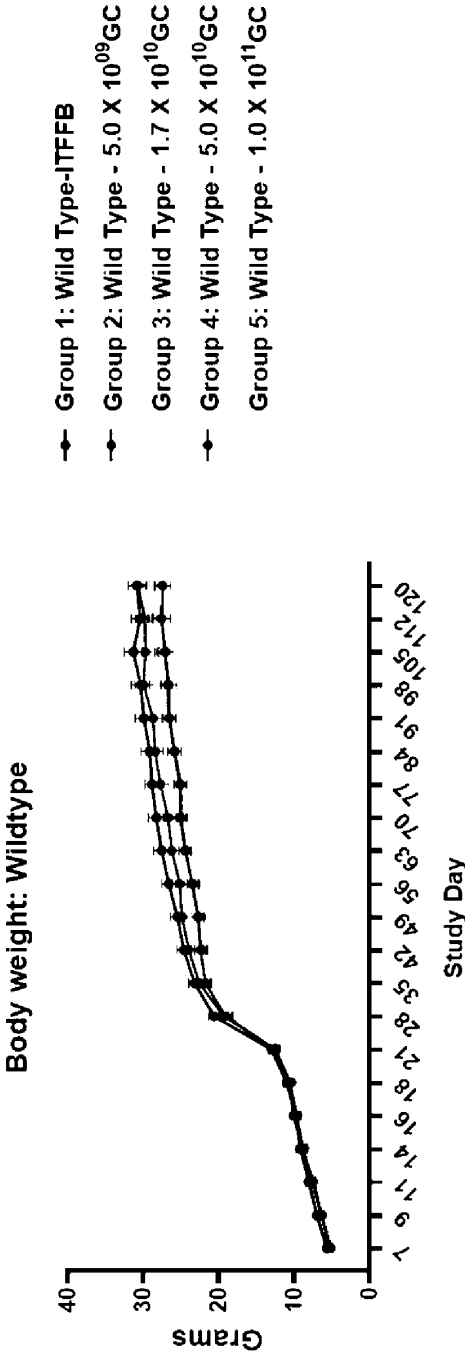
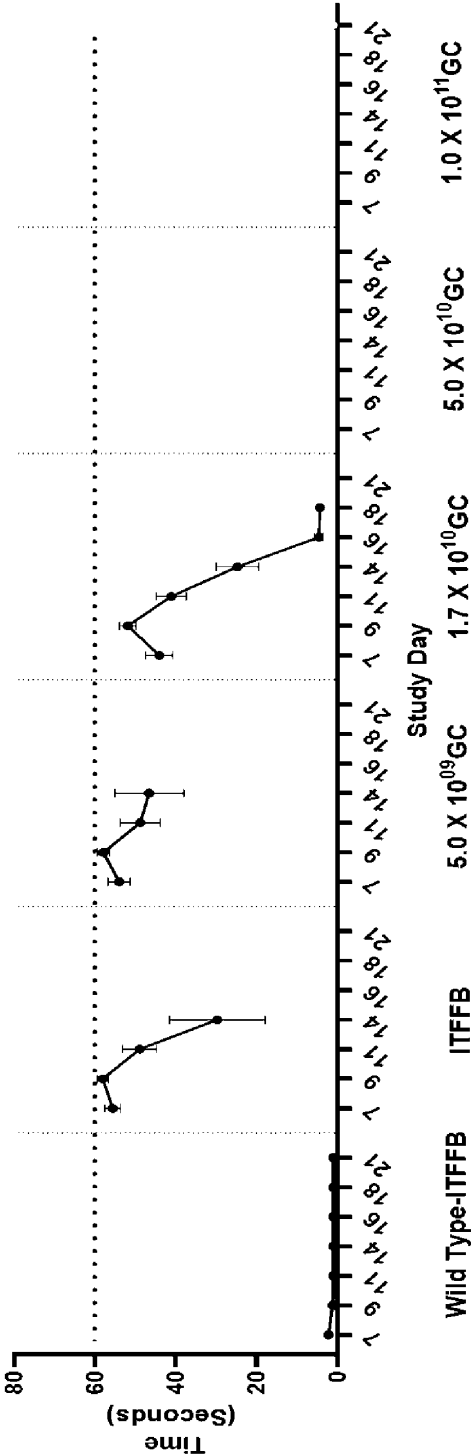


FIG. 18

FIG. 19

SMNΔ7: Righting Reflex



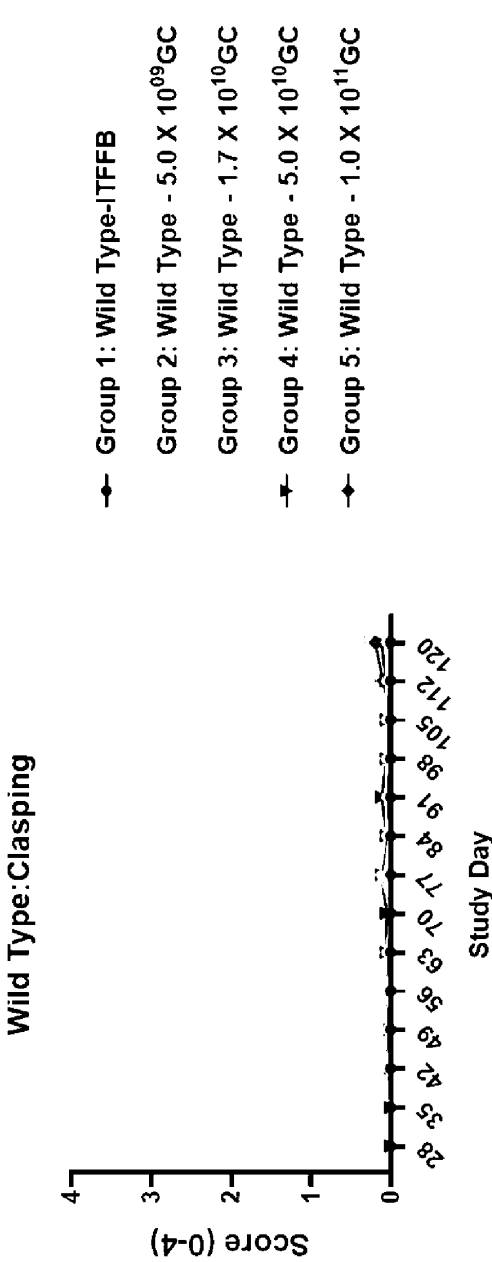


FIG. 20

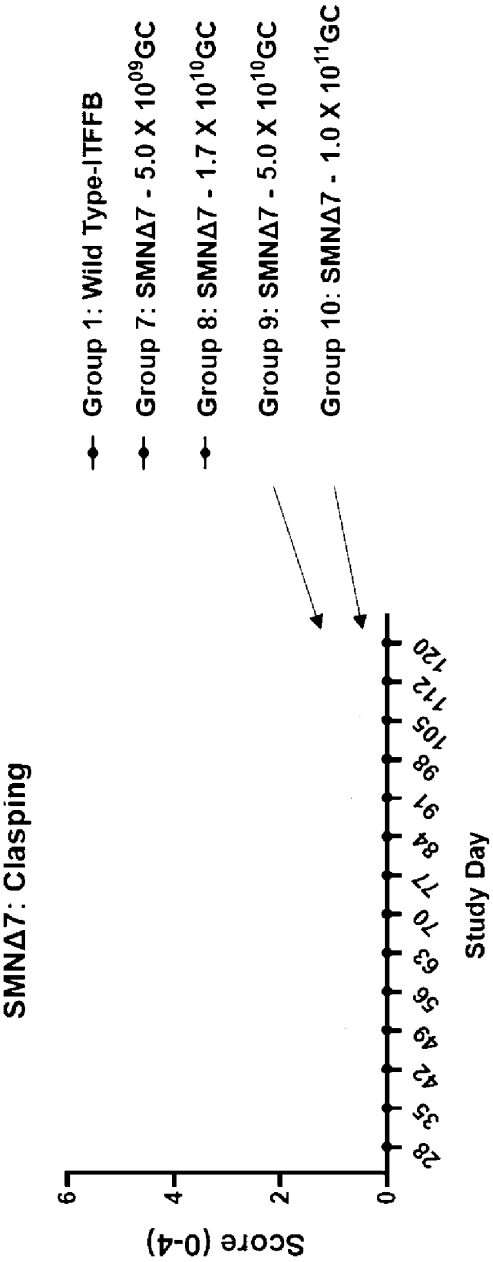


FIG. 21

FIG. 22

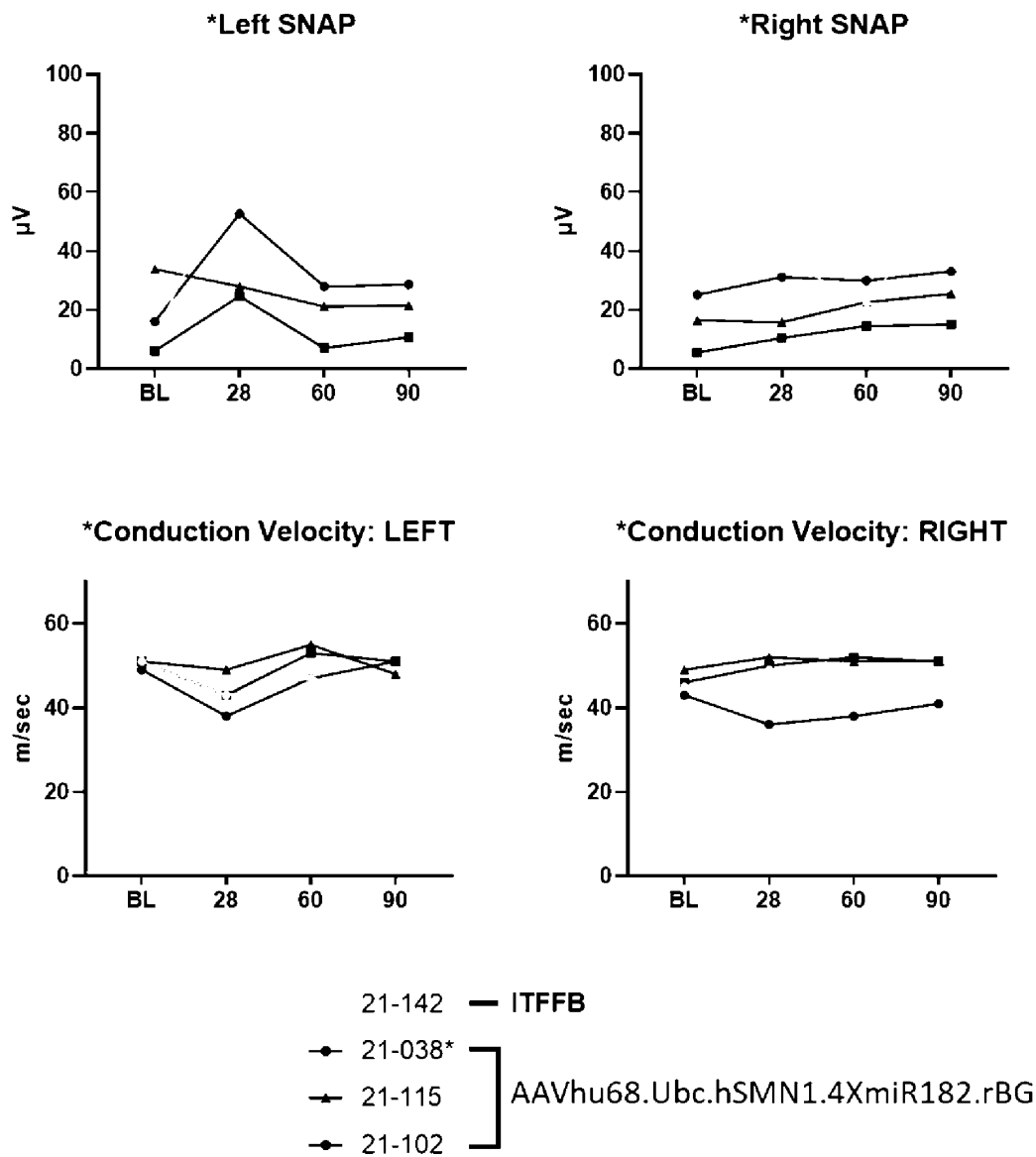


FIG. 23

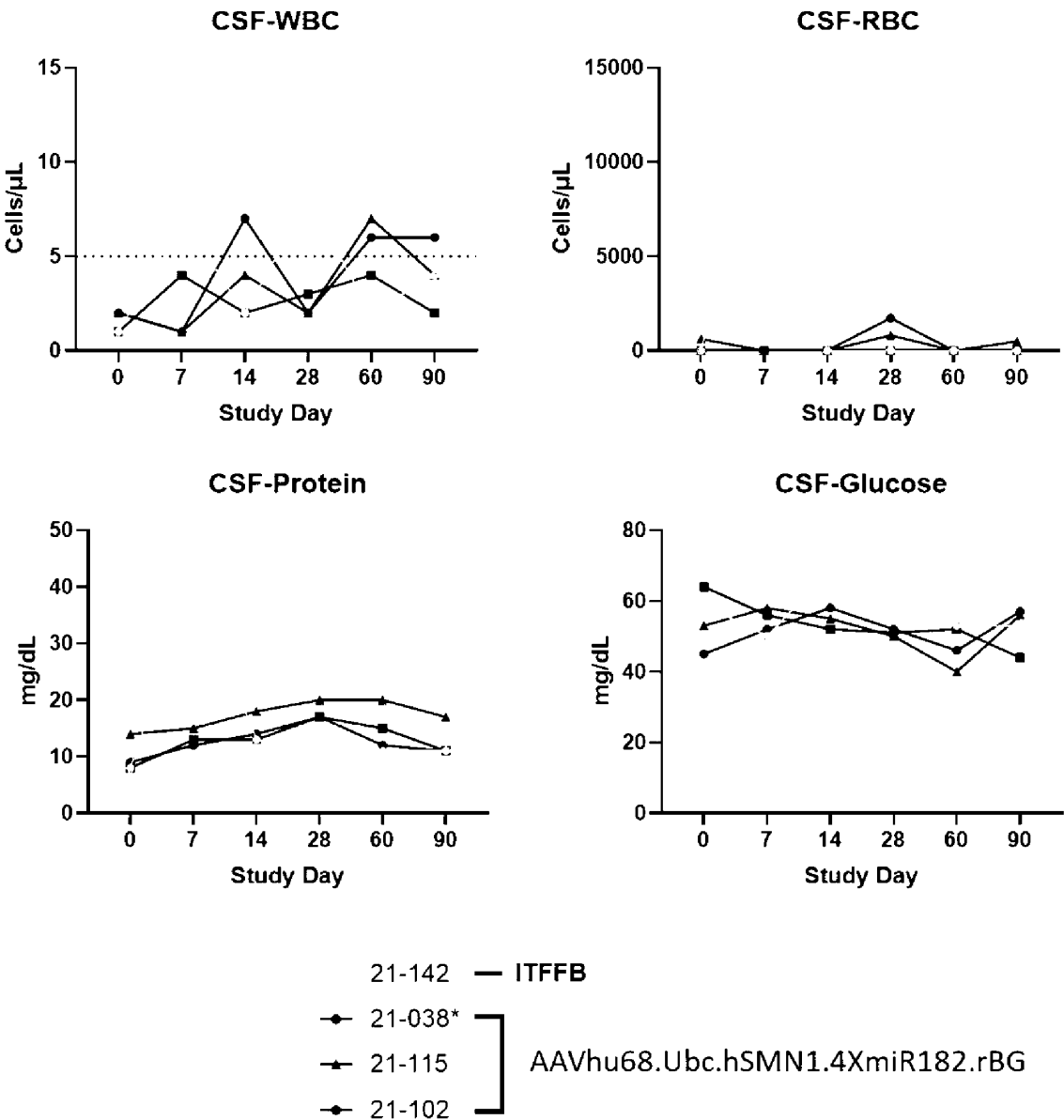


FIG. 24

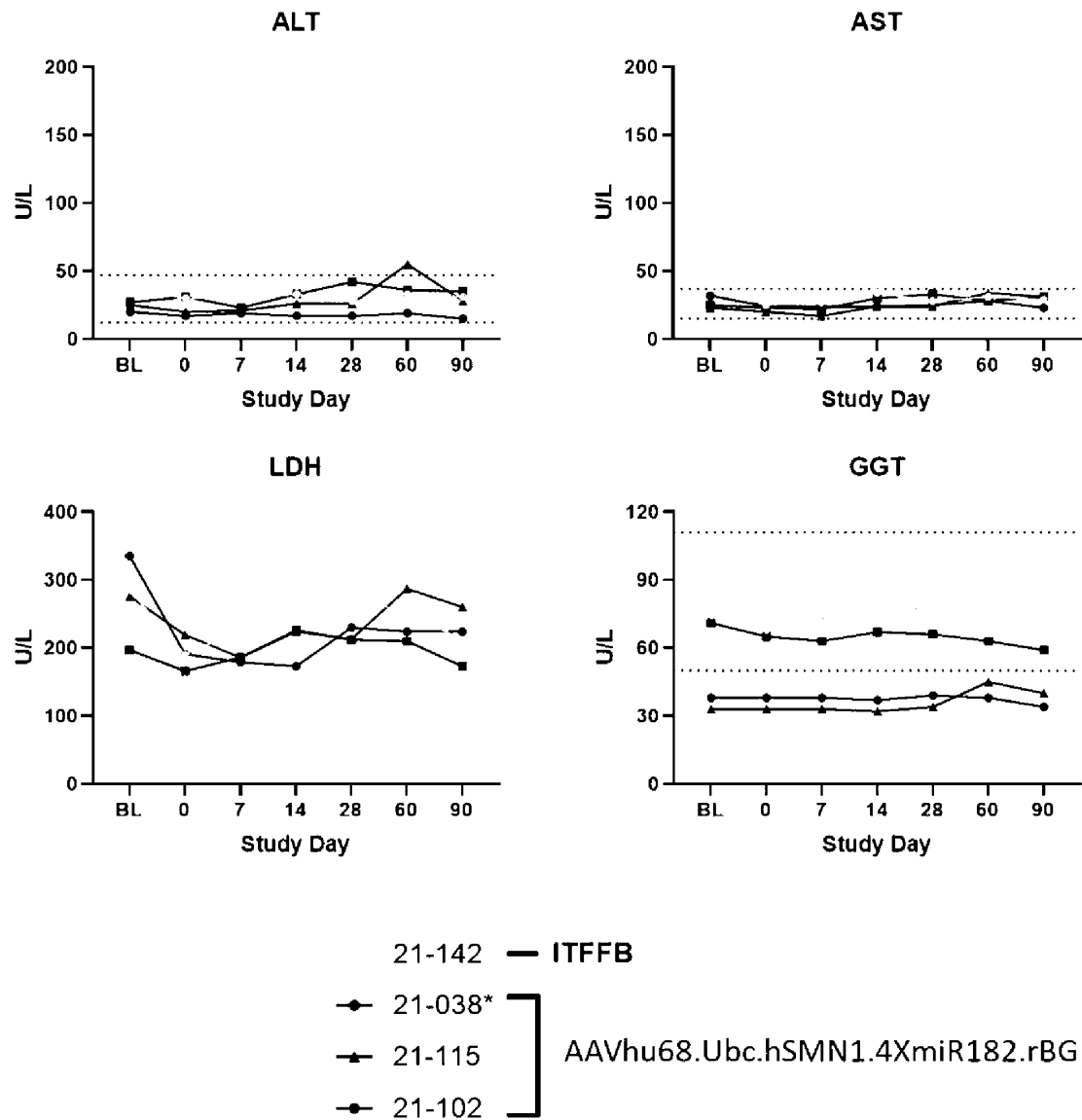
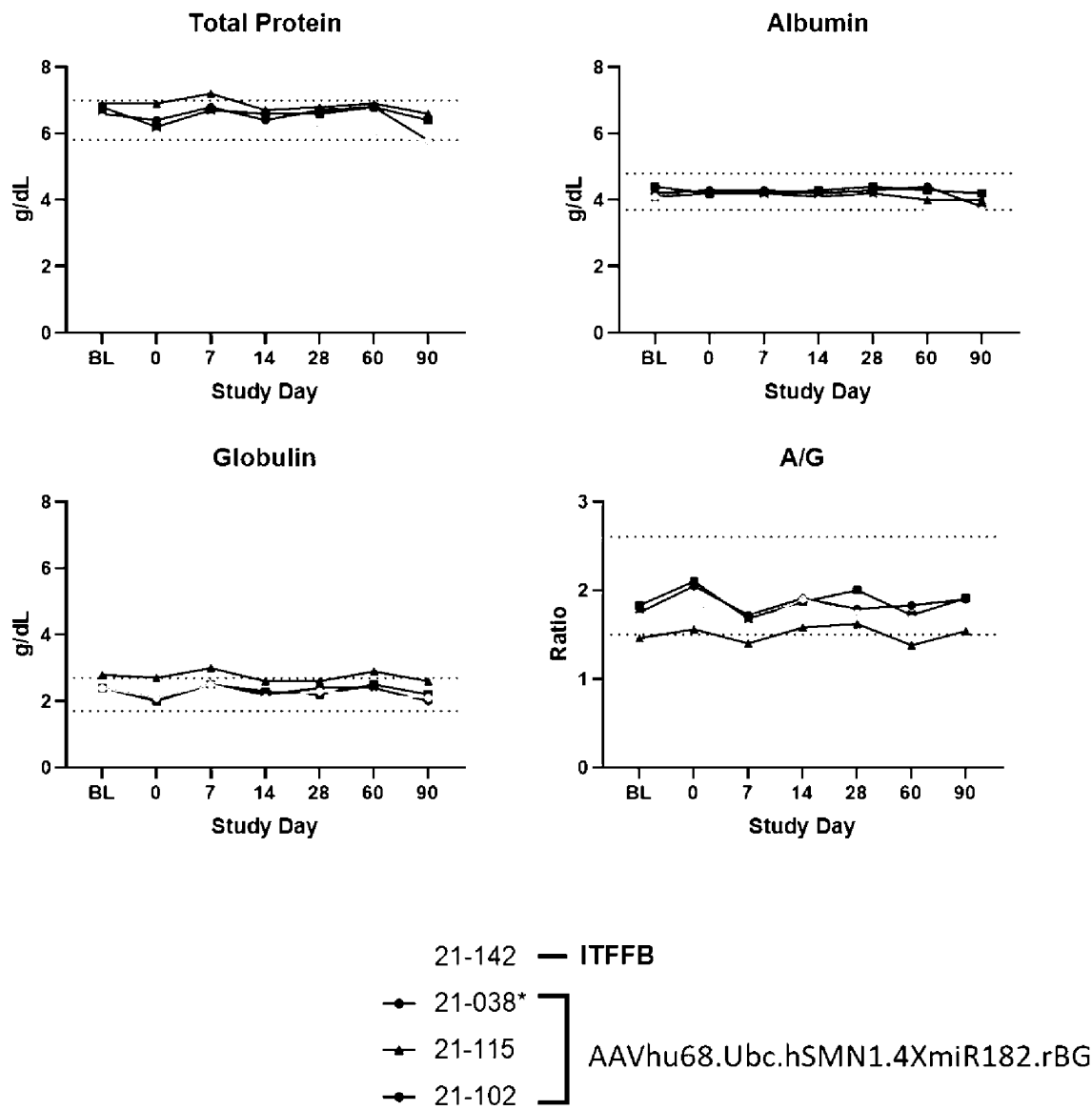


FIG. 25A



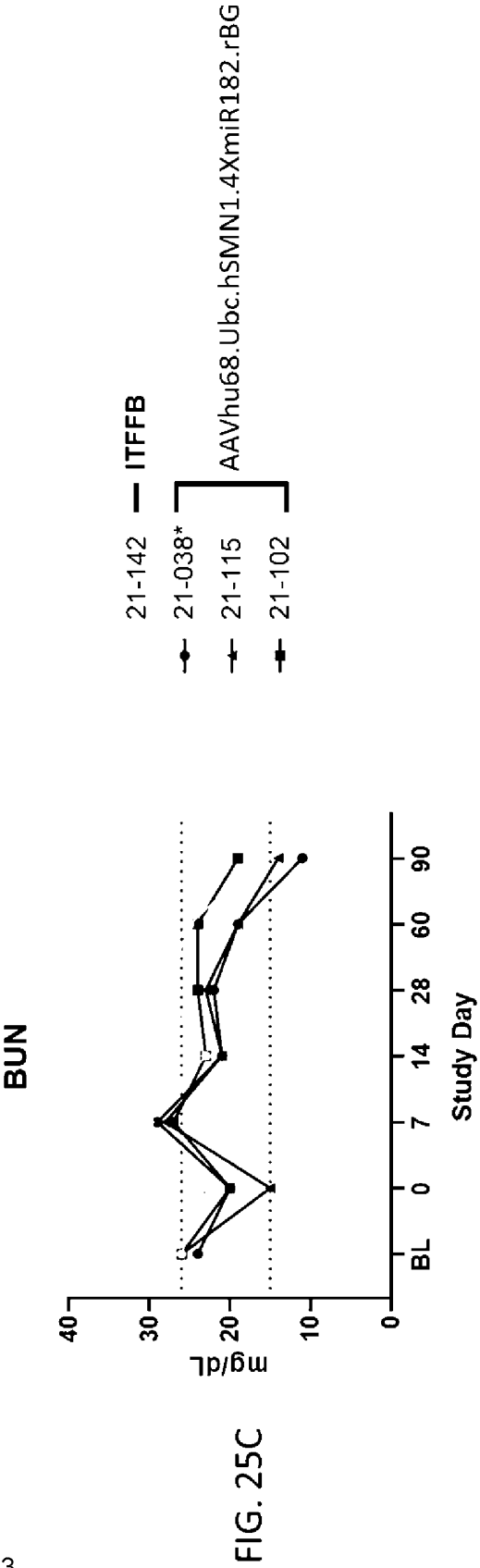
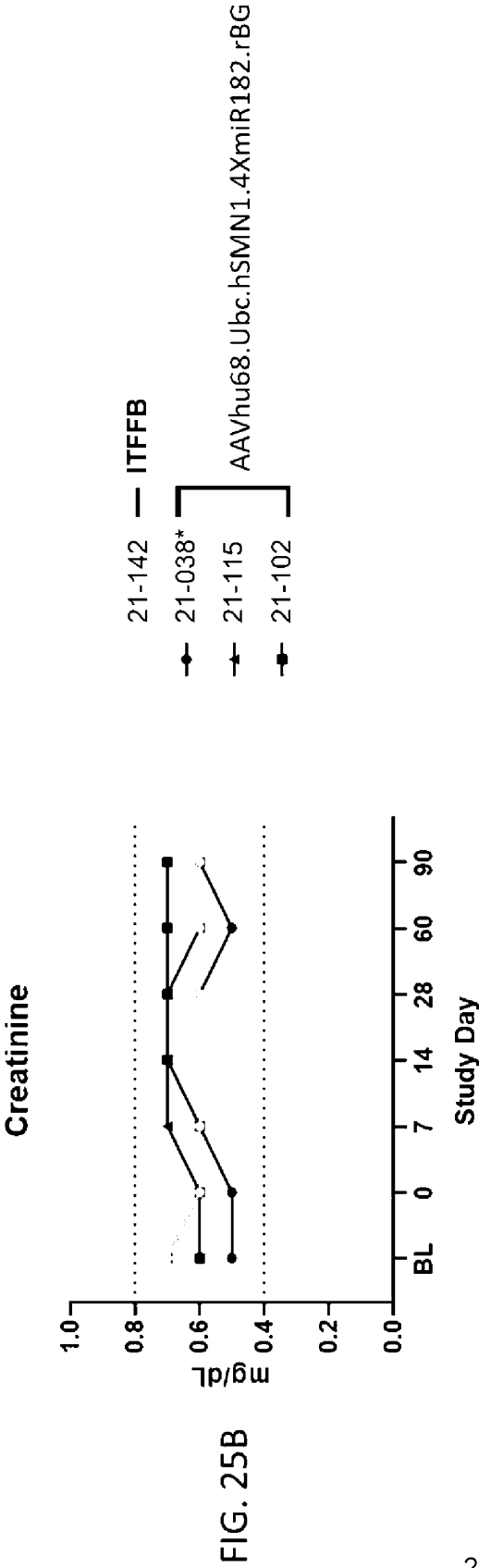
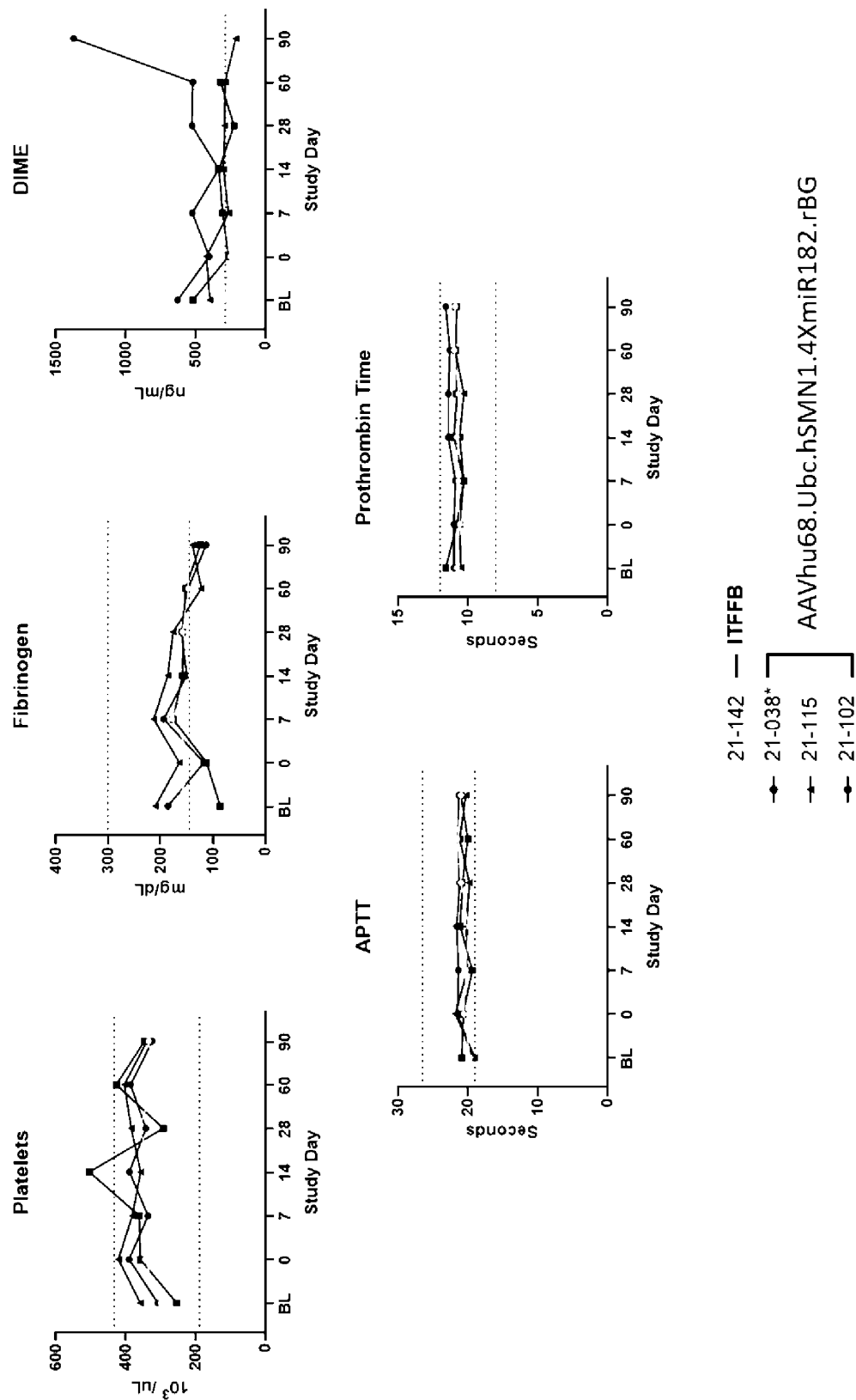


FIG. 26



WO 2025/035143

PCT/US2024/041840

INTERNATIONAL SEARCH REPORT

International application No

PCT/US2024/041840

A. CLASSIFICATION OF SUBJECT MATTER

INV. C07K14/44 C12N15/11 A61K38/00 C07K14/47 A61K48/00
C12N15/113 C12N15/86

ADD.

According to International Patent Classification (IPC) or to both national classification and IPC

B. FIELDS SEARCHED

Minimum documentation searched (classification system followed by classification symbols)

C07K C12N A61K

Documentation searched other than minimum documentation to the extent that such documents are included in the fields searched

Electronic data base consulted during the international search (name of data base and, where practicable, search terms used)

EPO-Internal

C. DOCUMENTS CONSIDERED TO BE RELEVANT

Category*	Citation of document, with indication, where appropriate, of the relevant passages	Relevant to claim No.
X	WO 2020/132455 A1 (UNIV PENNSYLVANIA [US]) 25 June 2020 (2020-06-25) see opinion for details -----	1 - 55
Y	WO 2023/056399 A1 (UNIV PENNSYLVANIA [US]) 6 April 2023 (2023-04-06) see opinion for details -----	1 - 55
Y	WO 2022/094078 A1 (UNIV PENNSYLVANIA [US]) 5 May 2022 (2022-05-05) see opinion for details -----	1 - 55

☐ Further documents are listed in the continuation of Box C.☒ See patent family annex.

* Special categories of cited documents :

"A" document defining the general state of the art which is not considered to be of particular relevance

"E" earlier application or patent but published on or after the international filing date

"L" document which may throw doubts on priority claim(s) or which is cited to establish the publication date of another citation or other special reason (as specified)

"O" document referring to an oral disclosure, use, exhibition or other means

"P" document published prior to the international filing date but later than the priority date claimed

"T" later document published after the international filing date or priority date and not in conflict with the application but cited to understand the principle or theory underlying the invention

"X" document of particular relevance; the claimed invention cannot be considered novel or cannot be considered to involve an inventive step when the document is taken alone

"Y" document of particular relevance; the claimed invention cannot be considered to involve an inventive step when the document is combined with one or more other such documents, such combination being obvious to a person skilled in the art

"&" document member of the same patent family

Date of the actual completion of the international search

Date of mailing of the international search report

7 November 2024

28/11/2024

Name and mailing address of the ISA/

European Patent Office, P.B. 5818 Patentlaan 2

NL - 2280 HV Rijswijk

Tel. (+31-70) 340-2040,

Fax: (+31-70) 340-3016

Authorized officer

Landré, Julien