

In Vivo-Targefect

A NON-VIRAL TRANSFECTION REAGENT FOR *IN VIVO* GENE TRANSFER TO SKELETAL MUSCLE

In vivo-Targefect is a proprietary sterile solution of a mixture of polymers which facilitate efficient, intramuscular delivery in vivo without any toxic effects. Gene expression is sustained for over a month and the expressed gene can be secreted from muscle cells using an appropriate secretory signal.

Advantages:

- Simple transfection protocol, no electroporation or pulsed electrical field.
- Sustained Gene transfer for at least 1 month
- Functionality validated in mice
- No detectable immune response
- Successful gene delivery via multiple routes of administration
- Efficient secretion of transgene from transduced muscle tissue using appropriate secretory signals

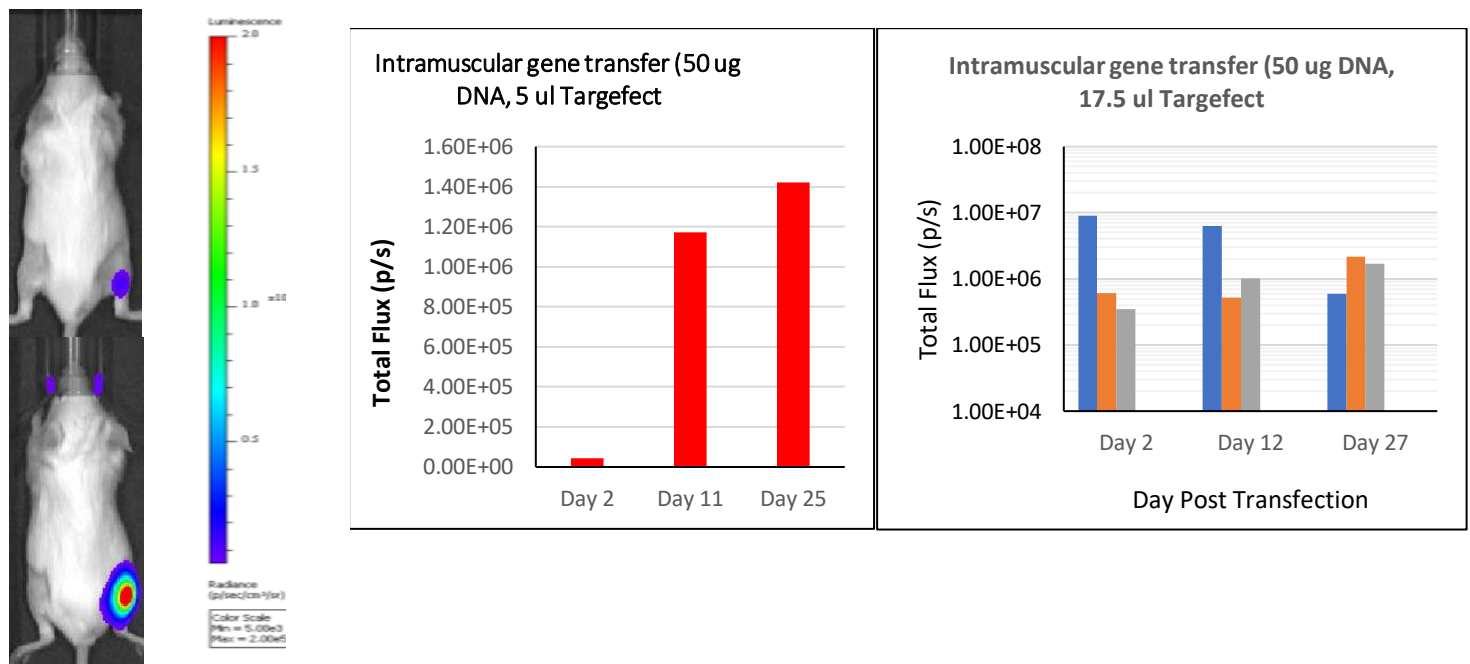


Figure 1: Intramuscular gene transfer of a luciferase expression vector, pCMV-RedFLuc using In vivo-Targefect: 50 ug of pCMV-RedFLuc plasmid DNA was complexed with 5 ul of In vivo-Targefect incubated at RT for 30 mins and injected into the right gastrocnemius (injection volume 15 ul). In vivo imaging was performed at day 2 and day 11 after injection using firefly luciferin on an ivis imaging system from Perkin Elmer). Left side of Figure shows representative BLI image.

In vivo gene transfer using a dual luciferase expressing lentiplasmid which co-expresses secreted Gaussia luciferase and an intracellular red-emitting firefly luciferase (Fluc): The Fluc is co-expressed using a T2a element so signal using a regular vector would be stronger. This plasmid was used because it co-expresses secreted Luc and intracellular Fluc, so the Fluc imaging helps establish gene expression at the site of injection and GLuc signal demonstrates secretability of the expressed gene. As seen from the data below the Gaussia luciferase gene is efficiently secreted.

Imaging of Gaussia luciferase expression in vivo following intramuscular injection:

Figure 2A: Whole body imaging with injected coelenterazine to demonstrate secretability of Gaussia luciferase from the transduced muscle tissue.

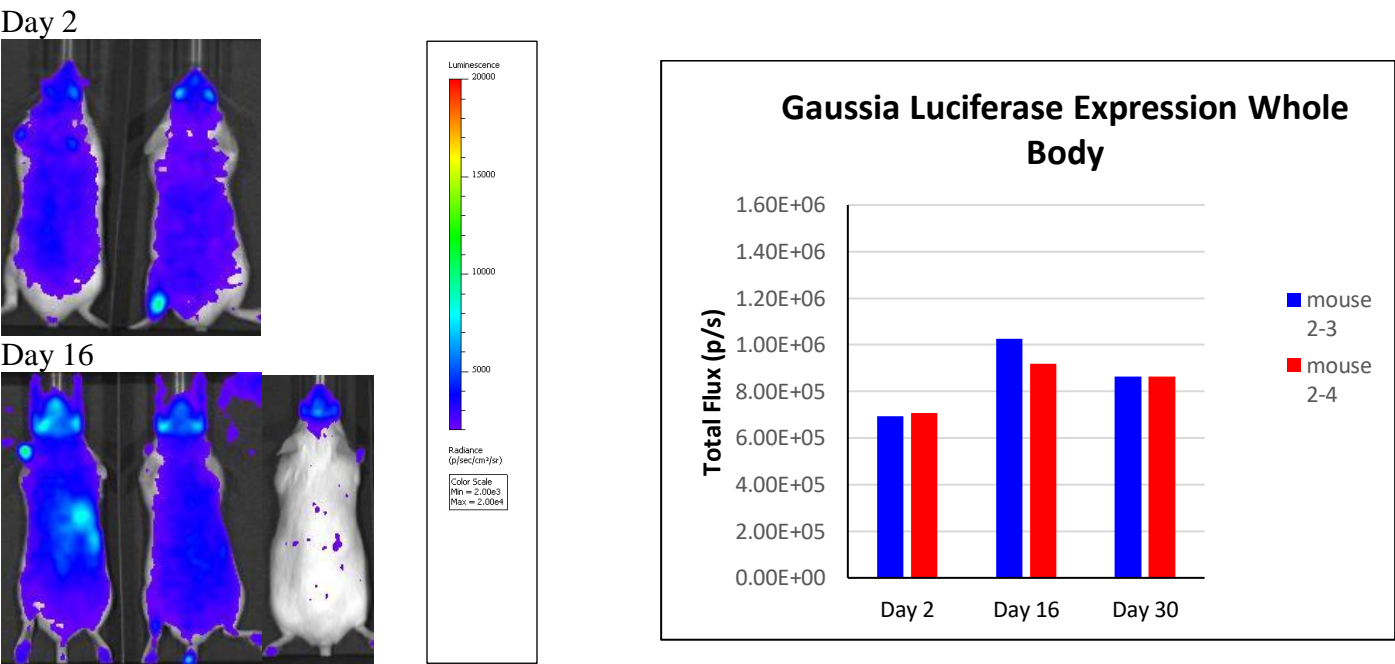
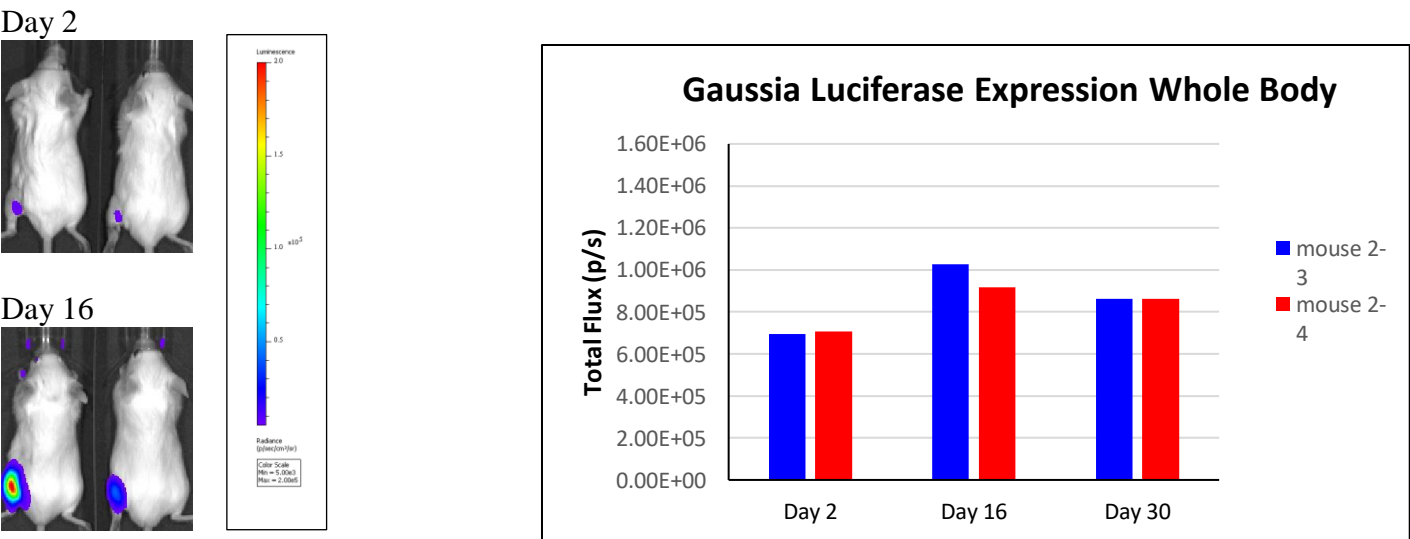


Figure 2B: Imaging of mice (shown in fig 2A) with firefly luciferin to demonstrate gene expression at site of injection



ORDERING INFORMATION:

| CATALOG NO. | SIZE | # INJECTIONS | PRICE |
|------------------|--------|----------------|--------|
| | | | |
| IN-VIVO-TARG-001 | 200 ul | 12 injections | \$600 |
| IN-VIVO-TARG-002 | 600 ul | 36 injections | \$1200 |
| IN-VIVO-TARG-003 | 3.2 ml | 180 injections | \$3500 |