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# Agrin therapy

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Congenital muscular dystrophy is a severe muscle-wasting disease that is often caused by mutations in LAMA2, the gene encoding the laminin α2 chain expressed by muscle fibres. In the September 20 Nature, Joachim Moll and colleagues at the University of Basel, Switzerland, report that an agrin minigene can rescue dystrophic symptoms in a mouse model of the disease (*Nature* 2001, **413:**302-307). The researchers reasoned that agrin, which binds to laminin and to α-drystroglycan, might be able to functionally rescue the weakened muscle caused by *LAMA2* mutations. They designed a truncated mini-agrin construct driven by the muscle creatine kinase promoter. They crossed mice expressing the mini-agrin transgene (mag-tg) with animals lacking a functional lama2gene. The agrin transgene improved the general health, lifespan and locomotory activity of the mutant mice. The agrin transgene also rescued the muscle degeneration phenotype. This study demonstrates the potential for gene therapy using non-homologous proteins that functionally compensate for gene mutation.

# References

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