

Bac

R a a a a a a c a ac b  
 ab a c c DNA c T a a b a c ca  
 a a N ab a c H a T  
 c b a a a a Ta c D a c  
 a ca c T c a  
 a G a a a a ca Ta  
 c a a T c a a a  
 T a ac c c a a b a a  
 a a a a c G a a a  
 b a a a a c T a c c  
 a a a a a c a  
 ab F T c a a a a  
 a c c a T a c  
 a a c a a c b a cab ac  
 c T a

W a

W a a c c c a c  
 ab a a a T c a ac a A  
 A ca V AAV c c ac T Ta a ac  
 b a c a a a c A a T a  
 AAV c a a a a a  
 c c T a Ta a a a c

S

AAV a a c c c c a a a  
 a c  
 CRISPR ba c  
 a c a a a c  
 M a c a a a a a  
 c ca a a T c a

O a a  
T c b a c a AAV a  
a ca  
T a ac b c ca a a ab a a  
a T a c

O c a a  
T c a b a a cab c  
a  
A a a a a a ab  
Da a c a c a a  
I a a a c a a  
a c c

W a c  
S a c  
S a c ASO a a c c a

Acc ab c a TRL L  
Ba c c b  
C c  
E a c c  
Va a ab c  
Va a a  
D a a  
R a a a  
P c c  
P c a

W a ca  
E b a  
S a c

B  
S R a c  
F a T K c a  
a

W a  
A Da c Sa T a ac ca a c aca c T  
a a b c a c a b c ac  
a b a a b c c a  
b a W b a ab a a b c a

a c c ab a O a c a a a  
b a ac a

## Reviewers

R S a  
S R a c

Ma a Na a c  
A c a D c

M Ka  
c

P a c ac U S F aT c T a c a  
b R McNa a c a