AKRON BIO

FREQUENTLY ASKED QUESTIONS

Recombinant NLS-SpCas9-NLS (Cas9) Nuclease Solution

Vials Cat. # PD1019-1000

DISCLAIMER: This product is currently under development and is available as Catalog # PD1019-1000 for research use only. Final cGMP product details are subject to change.

1. What is CRISPR?

CRISPR stands for Clustered Regularly Interspaced Short Palindromic Repeats. CRISPRs are critical components of the bacterial defense system, forming the basis of CRISPR-Cas9 genome editing. In genome engineering, the term "CRISPR" is often used as a reference to the various systems (e.g., Cas9) that can be programmed to target specific stretches of genetic code and to edit DNA at precise locations. With CRISPR technology, we can permanently modify the genes in living cells and organisms, making it possible to correct mutations in the human genome to treat previously untreatable diseases.

2. Where do CRISPRs come from?

CRISPRs are naturally occurring in archaea and bacteria, forming the backbone of their genetic defenses. These organisms capture pieces of invading viral DNA and use them to create DNA segments known as CRISPR arrays. The CRISPR arrays consist of repeating sequences of genetic code interrupted by "spacer" sequences. The spacer sequences are remnants of genetic code from previous viral invaders. The CRISPR arrays allow the bacteria to "remember" the viruses. If they attack again, the bacteria will produce RNA segments from the CRISPR arrays to target the viral DNA. The bacteria will then use Cas9 or a similar enzyme to cut the DNA apart, which finally disables the virus.

3. What is Cas9?

Cas9 (CRISPR associated protein 9), is a dual RNA-guided DNA endonuclease enzyme associated with the CRISPR adaptive immune system in *Streptococcus pyogenes*. *S. pyogenes* utilizes CRISPR to memorize invader DNA, and Cas9 to later challenge and cleave it. Cas9 challenges this invader DNA, unwinding the foreign DNA and checking for sites complementary to the 20 base-pair spacer regions of the guide RNA (gRNA). If the DNA substrate is complementary to the gRNA, Cas9 cleaves the invading DNA, subsequently disabling it.

4. What diseases are being targeted with CRISPR-Cas9 systems?

CRISPR forms the foundation of promising treatments for several genetic diseases, such as blood disorders (e.g., beta thalassemia & sickle-cell anemia); cancers (e.g., B-cell acute lymphoblastic leukemia, lung cancer, esophageal cancer, & multiple myeloma); and neurodegenerative disorders (e.g., Alzheimer's disease & Huntington's disease).

5. **Under what conditions should Akron's Cas9 Nuclease be stored?**
   Cas9 Nuclease should be stored at -80 °C. Akron's Cas9 is currently under a long-term stability program.

6. **Can the Akron Cas9 Nuclease undergo multiple freeze-thaw cycles?**
   Please avoid multiple freeze-thaw cycles.

7. **When does Akron expects to offer Cas9 Nuclease as a cGMP product?**
   Akron's cGMP Cas9 Nuclease product is currently under development. Please contact us for details and updates. The currently available PD1019-1000 product is for research use only.

For more information on our available products or for technical assistance, see contact info below.
For contract orders under master supply agreement, please inquire.