CRISPR Off-target Analysis Services

Advanced, end-to-end workflow from nomination to confirmation with orthogonality

Comprehensive end-to-end assessments using next-generation sequencing-based technology

Robust performance, rigorous analytical, and process standards for both nomination and confirmation assays

Expert regulatory support to navigate complex FDA and EMA requirements for IND/CTA submissions

Comprehensive reporting and technical consultations featuring prioritized off-target sites and recommended confirmation panels

Overview

Off-target effects can jeopardize the development of CRISPR-based therapies, making early detection essential for clinical success. Off-target analysis services from Integrated DNA Technologies (IDT) can identify and confirm unintended edits with high sensitivity, helping you meet regulatory expectations and advance confidently. Our proven approach has supported multiple IND approvals, accelerating the path from discovery to clinic.

Request a consultation

Case study: World's first mRNA-based personalized CRISPR therapy

Watch this on-demand webinar to learn how IDT enabled a successful eIND filing for a pediatric patient with Urea Cycle Disorder (UCD) using our orthogonal nomination and confirmation assays: **UNCOVERseq** and **rhAmpSeq™ CRISPR Analysis System**, respectively. Read the New England Journal of Medicine article **here**.

Watch now

Comprehensive analysis from discovery to IND

Our services include assays for both nomination and confirmation, using orthogonal methods from discovery through preclinical stages and IND filings.

FDA and other agencies	Nomination stage	Confirmation stage
Off-target nomination	Guide RNA selection, IND filings	**UNCOVERseq**: Identify potential off-target sites using an *in cellulo*, genome-wide, unbiased detection based on GUIDE-seq™ ***In silico* nomination**: Predict off-target regions using computational models
Off-target confirmation	Confirmation of off-target editing in cells, IND filings	**rhAmpSeq™ CRISPR Analysis System**: Detect small indels, base edits, and chromosomal translocations using high-throughput amplicon sequencing

Services aligned with guidance from FDA/EMA

Our services for both nomination and confirmation assays are aligned with both the FDA and EMA requirements on advanced therapies. Our dedicated regulatory team provides strategic guidance and documentation support to help you navigate regulatory requirements, streamline IND/CTA submission, and ensure alignment with evolving FDA and EMA expectations.

FDA and other agencies	Nomination stage	Confirmation stage
Utilize multiple complementary assessment methods	Yes	
Detect off-target effects with high analytical sensitivity	Yes	Yes
Analyze human cell types from multiple donors	Yes	Yes
Detail the biological impact of off-target effects	Yes	Yes

Our end-to-end process

Leverage our services from nomination to confirmation, or select individual services tailored to your specific needs. For greater flexibility, you can either send us your samples or have IDT generate the samples using in-house nomination model systems.

Sample preparation	• Option 1: Customer performs gene editing and sends cell pellet. • Option 2: IDT performs gene editing and generates cell pellet.
Off-target nomination	• IDT performs *in cellulo* UNCOVERseq assay and/or *in silico* nomination assay.
Report generation and review	• Provide off-target nomination report with recommended confirmation panel, including annotation of off-targets with functional risk data. • Conduct technical consultation for a detailed review of sites and recommendations and provide regulatory support for IND/CTA filings.
Off-target confirmation	• IDT designs and synthesizes rhAmpSeq panel. • IDT performs multiplexed rhAmpSeq assay. • IDT performs analysis to detect chromosomal translocations and small indels using PASTA and OTEasy, respectively.
Report generation and review	• Provide off-target confirmation report with annotation of off-target confirmation sites. • Conduct technical consultation with internal experts to review report and provide regulatory support for IND/CTA filings.

Ready to accelerate your CRISPR off-target analysis?

To learn more or request a consultation with our Clinical Development Leaders, visit **here**.

For more information, visit
idtdna.com/CRISPR-off-target-analysis-services

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