

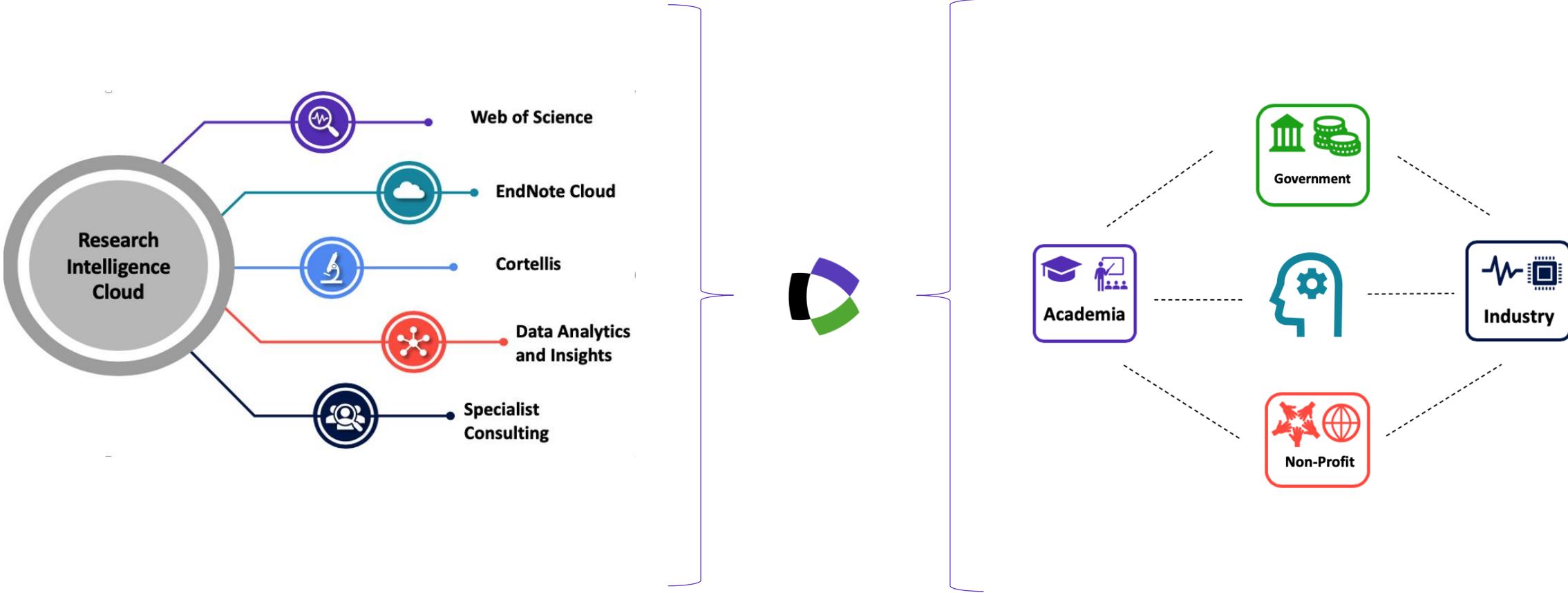
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Adriana FILIP - Solutions Consultant

adriana.filip@clarivate.com

Decembrie 2020

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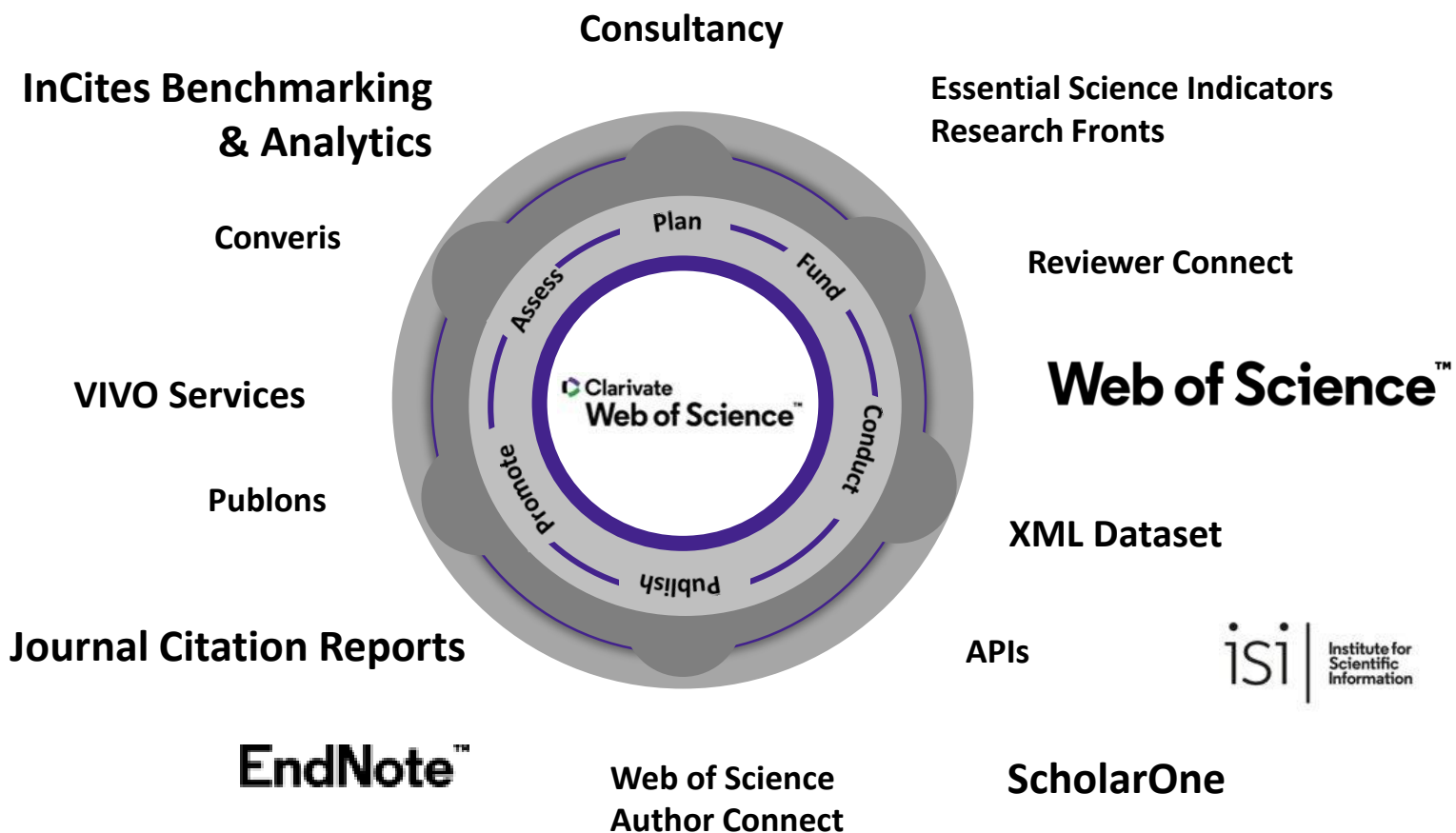


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 ANNALS OF PHYSICS Volume: 418 Article Number: 168180 Published: JUL 2020

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Scalable auto-encoders for gravitational waves detection from time series data

By: Corizzo, R [Corizzo, Roberto]^{1,2,4,1}; Ceci, M [Ceci, Michelangelo]^{2,4,5,1}; Zdravetski, E [Zdravetski, Eftim]^{3,1}; Japkowicz, N [Japkowicz, Nathalie]^{1,1}
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EXPERT SYSTEMS WITH APPLICATIONS
 Volume: 151
 Article Number: 113378
 DOI: 10.1016/j.eswa.2020.113378
 Published: AUG 1 2020
 Document Type: Article
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Abstract
 Gravitational waves represent a new opportunity to study and interpret phenomena from the universe. In order to efficiently detect and analyze them, advanced and automatic signal processing and machine learning techniques could help to support standard tools and techniques. Another challenge relates to the large volume of data collected by the detectors on a daily basis, which creates a gap between the amount of data generated and effectively analyzed. In this paper, we propose two approaches involving deep auto-encoder models to analyze time series collected from Gravitational Waves detectors and provide a classification label (noise or real signal). The purpose is to discard noisy time series accurately and identify time series that potentially contain a real phenomenon. Experiments carried out on three datasets show that the proposed approaches implemented using the Apache Spark framework, represent a valuable machine learning tool for astrophysical analysis, offering competitive accuracy and scalability performances with respect to state-of-the-art methods. (C) 2020 Elsevier Ltd. All rights reserved.

Keywords
 Author Keywords: Time series classification; Anomaly detection; Feature extraction; Deep neural networks; Machine learning; Big data analytics; Apache spark; Hadoop
 KeyWords Plus: CLASSIFICATION; ENSEMBLE; POWER

Author Information
 Corresponding Address: Corizzo, R (corresponding author)
 + Amer Univ, Dept Comp Sci, 4400 Massachusetts Ave NW, Washington, DC 20016 USA.
 Corresponding Address: Corizzo, R (corresponding author)
 + Univ Bari Aldo Moro, Dept Comp Sci, Via E Orabona 4, Bari 70125, Italy.
 Corresponding Address: Corizzo, R (corresponding author)
 Natl Interuniv Consortium Informat CINI, Via Volturmo 58, Rome 00185, Italy.

Addresses:
 + [1] Amer Univ, Dept Comp Sci, 4400 Massachusetts Ave NW, Washington, DC 20016 USA
 + [2] Univ Bari Aldo Moro, Dept Comp Sci, Via E Orabona 4, Bari 70125, Italy
 + [3] Ss Cyril & Methodius Univ, Fac Comp Sci & Engr, Rugjer Boshkovik 16, Skopje 1000, North Macedonia
 + [4] Natl Interuniv Consortium Informat CINI, Via Volturmo 58, Rome 00185, Italy
 + [5] Jozef Stefan Inst, Jamova 39, Ljubljana 1000, Slovenia

E-mail Addresses: rcorizzo@american.edu; michelangelo.ceci@uniba.it; eftim@fnki.ukim.mk; japkowicz@american.edu

Funding

Funding Agency	Show details	Grant Number
European Cooperation in Science and Technology (COST)		
Ministry of Education, Universities and Research (MIUR)		ARS01_01259
Ministry of Education, Universities and Research (MIUR)		ARS01_001413
Faculty of Computer Science and Engineering		

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1 How to talk about genome editing
Starr, S
Jun 2018 | British Medical Bulletin
43 References
Background: Human genome editing is an area of growing prominence, with many potential therapeutic applications. Sources of data: A project by two UK charities, whose participants included fertility sector patients and practitioners and also people affected by genetic disease and rare disease. Scientific ... Show more
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2 Improved CRISPR-mediated genome editing specificity with destabilized Cas9 (PEST-Cas9)
Liu, Y; Zhou, Y; (...); Luo, YL
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3 CRISPR clear? Dimeric Cas9-Fok1 nucleases improve genome-editing specificity
Zhang, HM; Yan, Z; (...); He, TC
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CRISPR-Cas9: A revolution in genome editing in rheumatic diseases

By: Duroux-Richard, J (Duroux-Richard, Isabelle) ¹; Giovannangeli, C (Giovannangeli, Carine) ²; Apparailly, F (Apparailly, Florence) ¹
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JOINT BONE SPINE
Volume: 84 Issue: 1 Pages: 1-4
DOI: 10.1016/j.jbspin.2016.09.012
Published: JAN 2017
Document Type: Editorial Material

Keywords
Author Keywords: CRISPR-Cas; Rheumatism; Genome editing
Keywords Plus: NUCLEASES; REPEATS; DNA; ACTIVATION

Author Information
Corresponding Address: Apparailly, Florence (corresponding author)
Univ Montpellier, CHRU Montpellier, INSERM, Clin Dept Osteoarticular Dis,U1183,IRMB, 80 Rue Augustin Fliche, F-34295 Montpellier, France
Addresses:
Univ Montpellier, CHRU Montpellier, INSERM, Clin Dept Osteoarticular Dis,U1183,IRMB, 80 Rue Augustin Fliche, F-34295 Montpellier, France
CNRS, INSERM, U1154,UMR 7196, Museum Natl Hist Nat Struct & Instabilite Genomes, 43 Rue Cuvier, F-75231 Paris, France
E-mail Addresses: florence.apparailly@inscm.fr

Categories/Classification
Research Areas: Rheumatology

Funding

Funding agency	Show details	Grant number
Institut National de la Sante et de la Recherche Medicale (Inserm)		
National Museum of Natural History in Paris, France		
Montpellier university		

Close funding text
This work was supported by the Inserm, Montpellier university, and National Museum of Natural History in Paris, France.

Document Information
Accession Number: WOS:000396443800001
PubMed ID: 27825565
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ISSN: 1297-319X
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Current Publisher: ELSEVIER FRANCE-EDITIONS SCIENTIFIQUES MEDICALES ELSEVIER, 65 RUE CAMILLE DESMOULINS, CS50083, 92442 ISSY-LES-MOULINEAUX, FRANCE
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Research Areas: Rheumatology
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3.741
Journal impact factor (2019)

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Mglinets, V.A.; Мглинец, В.А.; Therapeutic approaches of the CRISPR/Cas genome editing system for genetic diseases in humans and model animals Терапевтические подходы к использованию системы редактирования генома CRISPR/Cas при наследственных болезнях у человека и модельных животных Meditsinskaya genetika Медицинская генетика

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Reduction sensitive linkers (RSLs) have the potential to transform the field of drug delivery due to their ease of use and selective cleavage in intracellular environments. However, despite their compelling attributes, developing reduction sensitive self-immolative linkers for aliphatic amines has been challenging due to their poor leaving group ability and high pK(a) values. Here a traceless self-immolative linker composed of a dithiol-ethyl carbonate connected to a benzyl carbamate (DEC) is presented, which can modify aliphatic amines and release them rapidly and quantitatively after disulfide reduction. DEC was able to reversibly modify the lysine residues on CRISPR-Cas9 with either PEG, the cell penetrating peptide Arg(10), or donor DNA, and generated Cas9 conjugates with significantly improved biological properties. In particular, Cas9-DEC-PEG was able to diffuse through brain tissue significantly better than unmodified Cas9, making it a more suitable candidate for genome editing in animals. Furthermore, conjugation of Arg(10) to Cas9 with DEC was able to generate a self-delivering Cas9 RNP that could edit cells without transfection reagents. Finally, conjugation of donor DNA to Cas9 with DEC increased the homology directed DNA repair (HDR) rate of the Cas9 RNP by 50% in HEK 293T cell line. We anticipate that DEC will have numerous applications in biotechnology, given the ubiquitous presence of aliphatic amines on small molecule and protein therapeutics.

Keywords

KeyWords Plus: DRUG; PEGYLATION; DELIVERY; CONJUGATION; POLYMERS; PRODRUGS; PROTEIN

Author Information

Reprint Address:

University of California System University of California Berkeley Univ Calif Berkeley, Dent Bldg, Univ Ave, Berkeley, CA 94720 USA

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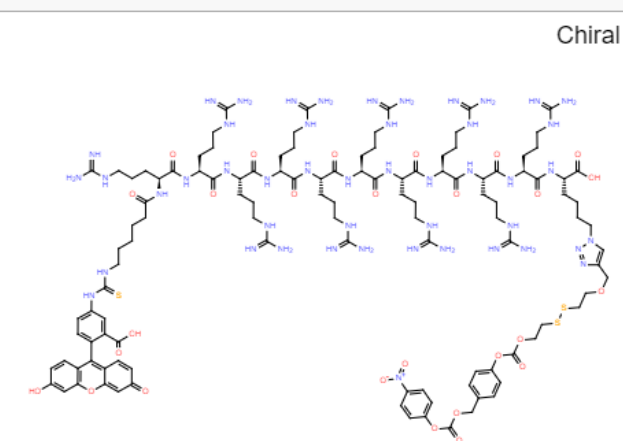
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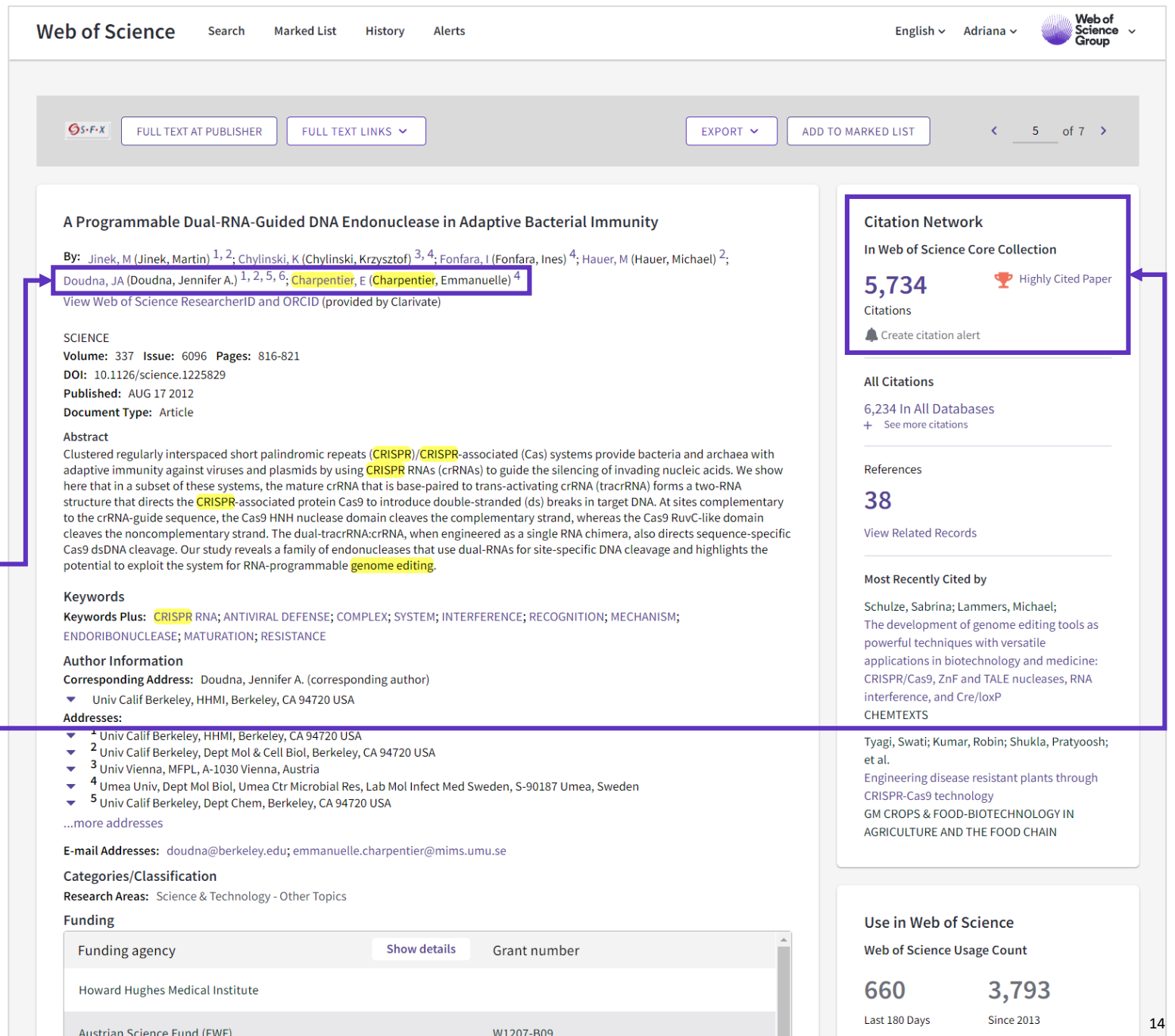
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Identify Top Papers

Emmanuelle Charpentier and Jennifer Doudna share the **2020 Nobel chemistry prize** for developing the precise genome-editing technology.

Highly Cited Paper published in 2012.



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A Programmable Dual-RNA-Guided DNA Endonuclease in Adaptive Bacterial Immunity

By: Jinek, M (Jinek, Martin)^{1, 2}; Chylinski, K (Chylinski, Krzysztof)^{3, 4}; Fonfara, I (Fonfara, Ines)⁴; Hauer, M (Hauer, Michael)²; Doudna, JA (Doudna, Jennifer A.)^{1, 2, 5, 6}; Charpentier, E (Charpentier, Emmanuelle)⁴
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SCIENCE
Volume: 337 Issue: 6096 Pages: 816-821
DOI: 10.1126/science.1225829
Published: AUG 17 2012
Document Type: Article

Abstract
Clustered regularly interspaced short palindromic repeats (CRISPR)/CRISPR-associated (Cas) systems provide bacteria and archaea with adaptive immunity against viruses and plasmids by using CRISPR RNAs (crRNAs) to guide the silencing of invading nucleic acids. We show here that in a subset of these systems, the mature crRNA that is base-paired to trans-activating crRNA (tracrRNA) forms a two-RNA structure that directs the CRISPR-associated protein Cas9 to introduce double-stranded (ds) breaks in target DNA. At sites complementary to the crRNA-guide sequence, the Cas9 HNH nuclease domain cleaves the complementary strand, whereas the Cas9 RuvC-like domain cleaves the noncomplementary strand. The dual-tracrRNA:crRNA, when engineered as a single RNA chimera, also directs sequence-specific Cas9 dsDNA cleavage. Our study reveals a family of endonucleases that use dual-RNAs for site-specific DNA cleavage and highlights the potential to exploit the system for RNA-programmable genome editing.

Keywords
Keywords Plus: CRISPR RNA; ANTIVIRAL DEFENSE; COMPLEX; SYSTEM; INTERFERENCE; RECOGNITION; MECHANISM; ENDORIBONUCLEASE; MATURATION; RESISTANCE

Author Information
Corresponding Address: Doudna, Jennifer A. (corresponding author)
Univ Calif Berkeley, HHMI, Berkeley, CA 94720 USA
Addresses:
1 Univ Calif Berkeley, HHMI, Berkeley, CA 94720 USA
2 Univ Calif Berkeley, Dept Mol & Cell Biol, Berkeley, CA 94720 USA
3 Univ Vienna, MFPL, A-1030 Vienna, Austria
4 Umea Univ, Dept Mol Biol, Umea Ctr Microbial Res, Lab Mol Infect Med Sweden, S-90187 Umea, Sweden
5 Univ Calif Berkeley, Dept Chem, Berkeley, CA 94720 USA
...more addresses

E-mail Addresses: doudna@berkeley.edu; emmanuelle.charpentier@mims.umu.se


Categories/Classification
Research Areas: Science & Technology - Other Topics

Funding

Funding agency	Show details	Grant number
Howard Hughes Medical Institute		
Austrian Science Fund (FWF)		W1207-B09

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Schulze, Sabrina; Lammers, Michael;
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Jennifer A. Doudna	Emmanuelle Charpentier
Professor of Molecular and Cell Biology and of Chemistry, University of California Berkeley, Berkeley, CA, United States; and Howard Hughes Medical Institute Investigator	Associate Professor, Laboratory for Molecular Infection Medicine Sweden (MIMS, Swedish Node of the European Molecular Biology Laboratory [EMBL] Partnership for Molecular Medicine), Umeå University, Umeå, Sweden; Professor, Hannover Medical School, Hannover, Germany; and, Head, Department Regulation in Infection Biology, Helmholtz Centre for Infection Research, Braunschweig, Germany
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Successful predictions by Nobel Prize winners: since 2002, our analysis has identified 59 scientists who have won the Nobel Prize.

Jennifer A. Doudna – Facts – 2020. NobelPrize.org. Nobel Media AB 2020. Wed. 2 Dec 2020. <https://www.nobelprize.org/prizes/chemistry/2020/doudna/facts/>

Emmanuelle Charpentier – Facts – 2020. NobelPrize.org. Nobel Media AB 2020. Wed. 2 Dec 2020. <https://www.nobelprize.org/prizes/chemistry/2020/charpentier/facts/>

Jennifer A. Doudna Facts



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Jennifer A. Doudna
The Nobel Prize in Chemistry 2020

Born: 19 February 1964, Washington, DC, USA

Affiliation at the time of the award: University of California, Berkeley, CA, USA

Prize motivation: "for the development of a method for genome editing."

Prize share: 1/2

Emmanuelle Charpentier Facts



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Emmanuelle Charpentier
The Nobel Prize in Chemistry 2020

Born: 11 December 1968, Juvisy-sur-Orge, France

Affiliation at the time of the award: Max Planck Unit for the Science of Pathogens, Berlin, Germany

Prize motivation: "for the development of a method for genome editing."

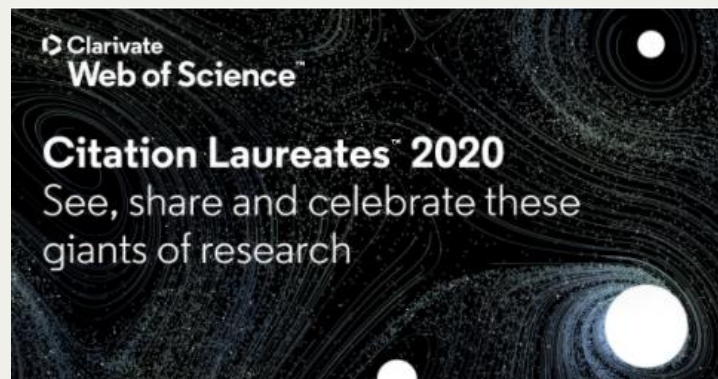
Prize share: 1/2

Researcher Recognition

<https://clarivate.com/webofsciencegroup/solutions/researcher-recognition/>



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A Programmable Dual-RNA-Guided DNA Endonuclease in Adaptive Bacterial Immunity


By: Jinek, M (Jinek, Martin)^{1, 2}; Chylinski, K (Chylinski, Krzysztof)^{3, 4}; Fonfara, I (Fonfara, Ines)⁴; Hauer, M (Hauer, Michael)²; Doudna, JA (Doudna, Jennifer A.)^{1, 2, 5, 6}; Charpentier, E (Charpentier, Emmanuelle)⁴

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Hauer, Michael	U-4800-2019	http://orcid.org/0000-0001-7463-3191
Jinek, Martin	E-6621-2011	http://orcid.org/0000-0002-7601-210X
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Bio
Michael Hauer has not yet added a bio to their profile.

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Michael Hauer has not yet added any institutions to their profile.

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A Programmable Dual-RNA-Guided DNA Endonuclease in Adaptive Bacterial Immunity

By: Jinek, M (Jinek, Martin)^{1, 2}; Chylinski, K (Chylinski, Krzysztof)^{3, 4}; Fonfara, I (Fonfara, Ines)⁴; Hauer, M (Hauer, Michael)²; Doudna, JA (Doudna, Jennifer A.)^{1, 2, 5, 6}; Charpentier, E (Charpentier, Emmanuelle)⁴
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SCIENCE
Volume: 337 Issue: 6096 Pages: 816-821
DOI: 10.1126/science.1225829
Published: AUG 17 2012
Document Type: Article

Abstract
Clustered regularly interspaced short palindromic repeats (CRISPR)/CRISPR-associated (Cas) systems provide bacteria and archaea with adaptive immunity against viruses and plasmids by using CRISPR RNAs (crRNAs) to guide the silencing of invading nucleic acids. We show here that in a subset of these systems, the mature crRNA that is base-paired to trans-activating crRNA (tracrRNA) forms a two-RNA structure that directs the CRISPR-associated protein Cas9 to introduce double-stranded (ds) breaks in target DNA. At sites complementary to the crRNA-guide sequence, the Cas9 HNH nuclease domain cleaves the complementary strand, whereas the Cas9 RuvC-like domain cleaves the noncomplementary strand. The dual-tracrRNA:crRNA, when engineered as a single RNA chimera, also directs sequence-specific Cas9 dsDNA cleavage. Our study reveals a family of endonucleases that use dual-RNAs for site-specific DNA cleavage and highlights the potential to exploit the system for RNA-programmable genome editing.

Keywords
Keywords Plus: CRISPR RNA; ANTIVIRAL DEFENSE; COMPLEX; SYSTEM; INTERFERENCE; RECOGNITION; MECHANISM; MUTATION; RESISTANCE

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Corresponding Address: Doudna, Jennifer A. (corresponding author)

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Schulze, Sabrina; Lammers, Michael;
The development of genome editing tools as powerful techniques with versatile applications in biotechnology and medicine: CRISPR/Cas9, ZnF and TALE nucleases, RNA

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Relevance

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1

1 How to talk about genome editing

Starr, S

Jun 2018 | British Medical Bulletin

Background: Human genome editing is an area of growing prominence, with many potential therapeutic applications. Sources of data: A project by two UK charities, whose participants included fertility sector patients and practitioners and also people affected by genetic disease and rare disease. Scienc... [Show more](#)

43

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 JAMA Neurol. 2018; November 01; 15(11): 1349-1355. doi:10.1001/jamaneurol.2018.1308.
 Published in final edited form as:
 JAMA Neurol. 2018; November 01; 15(11): 1349-1355. doi:10.1001/jamaneurol.2018.1308.

Genome Editing of Monogenic Neuromuscular Diseases: A Systematic Review
 Chingta Long, PhD, Leonia Amemiya, PhD, Rhonda Bassel-Duby, PhD, and Eric N. Olson, PhD
 Department of Molecular Biology, Senator Paul D. Wellstone Muscular Dystrophy Cooperative Research Center and Hamner Center for Regenerative Science and Medicine, The University of Texas Southwestern Medical Center, Dallas.

Abstract
IMPORTANCE—Muscle weakness, the most common symptom of neuromuscular disease, may result from muscle dysfunction or may be caused indirectly by neuronal and neuromuscular junction abnormalities. In dogs, more than 180 monogenic neuromuscular diseases, linked to 417 different genes, have been identified in humans. Genome-editing methods, especially the CRISPR/Cas9 (clustered regularly interspaced short palindromic repeat) Cas9/CRISPR-associated protein 9 system, hold clinical potential for curing many monogenic diseases, including neuromuscular diseases such as Duchenne muscular dystrophy, spinal muscular atrophy, amyotrophic lateral sclerosis, and myotonic dystrophy type 1.

OBJECTIVES—To provide an overview of genome-editing approaches, to summarize published reports on the feasibility, efficacy, and safety of current genome-editing methods as they relate to the potential correction of monogenic neuromuscular diseases, and to highlight scientific and clinical opportunities and obstacles toward permanent correction of disease-causing mutations responsible for monogenic neuromuscular disease by genome editing.

EVIDENCE REVIEW—PubMed and Google Scholar were searched for articles published from June 30, 1999, through June 9, 2018, using the following keywords: genome editing, CRISPR/Cas9, neuromuscular disease, Duchenne muscular dystrophy, spinal muscular atrophy, amyotrophic lateral sclerosis, and myotonic dystrophy type 1. The following sources were reviewed: 141 articles describing different approaches to all neuromuscular diseases; 139 articles describing CRISPR/Cas9-mediated genome editing in cell culture lines (in vitro) and animal models.

Concluding Author Note: Dr. Olson, PhD, Department of Molecular Biology, Senator Paul D. Wellstone Muscular Dystrophy Cooperative Research Center and Hamner Center for Regenerative Science and Medicine, The University of Texas Southwestern Medical Center, 6000 Burnet Road, Dallas, TX 75390; also lead author for all sections of this review and the responsibility for the integrity of the review and accuracy of the information.
 Author Contributions: Dr. Long and Dr. Olson conceived the review and were responsible for the majority of the review and accuracy of the information.
 Acquisition, analysis, or interpretation of data: Long, Amemiya, Olson.
 Drafting of the manuscript: Long, Amemiya, Olson.
 Approval of the manuscript for publication: Long, Amemiya, Olson.
 All authors approved the final manuscript.
 Address correspondence to: Dr. Eric N. Olson, Department of Molecular Biology, Senator Paul D. Wellstone Muscular Dystrophy Cooperative Research Center and Hamner Center for Regenerative Science and Medicine, The University of Texas Southwestern Medical Center, 6000 Burnet Road, Dallas, TX 75390; also lead author for all sections of this review and the responsibility for the integrity of the review and accuracy of the information.
 Address correspondence to: Dr. Eric N. Olson, Department of Molecular Biology, Senator Paul D. Wellstone Muscular Dystrophy Cooperative Research Center and Hamner Center for Regenerative Science and Medicine, The University of Texas Southwestern Medical Center, 6000 Burnet Road, Dallas, TX 75390; also lead author for all sections of this review and the responsibility for the integrity of the review and accuracy of the information.

genes

Genome Editing Tools in Plants
 Review
 Tayan Kumar Mohanta^{1,2}, Tefali Baslin¹, Akbar Hasbani^{1,3}, Elayed Fahil Abd, ALHA⁴ and Hisham Bay^{5,6*}

Abstract
 Genome editing tools have the potential to change the genetic architecture of a genome at precise locations, with desired accuracy. These tools have been successfully used for trait discovery and for the generation of plants with high crop yields and resistance to biotic and abiotic stresses. Due to complex genetic architecture, it is challenging to edit all of the genes/genomes using a particular genome editing tool. Therefore, to overcome this challenging task, several genome editing tools have been developed to facilitate efficient genome editing. Some of the major genome editing tools used to edit plant genomes are: Homologous recombination (HR), zinc finger nucleases (ZFNs), transcription activator-like effector nucleases (TALENs), protein-protein repeat primers (PPRs), the CRISPR/Cas9 system, RNA interference (RNAi), oligonucleotides, and oligonucleotides. In addition, site-directed sequence editing and oligonucleotide-directed mutagenesis have the potential to edit the genome at the single nucleotide level. Recently, adenine base editors (ABEs) have been developed to mutate A-T base pairs to G-C base pairs. ABEs use deaminase/transferase (DA) with catalytically impaired Cas9 nucleases to mutate A-T base pairs to G-C base pairs.

Keywords: genome editing, homologous recombination, Zinc finger nucleases, TALEN, protein-protein repeat primers, CRISPR/Cas9, adenine base editors, RNAi, site-directed sequence editing, oligonucleotide-directed mutagenesis, oligonucleotides and oligonucleotide-directed genome editing.

1. Introduction
 Since the beginning of plant domestication approximately 10,000 years ago, conventional plant breeding methods were the most successful approach for developing new crop varieties. Conventional plant breeding has combined extensively towards finding the wild and has played crucial roles in the development of modern society. The genetic breeding programs have led to the development of semi-dwarf and high-yielding crop varieties. Breeding programs of the past century have relied on natural and mutant-induced genetic variations to select for favorable genetic contributions. The traditional breeding program that is conducted by manipulating using chemical mutagenesis or irradiation, followed by screening for desired mutations, has several drawbacks. Methods using mutagenesis, interspecific crosses, and translocation breeding are non-specific and sometimes large parts of the genome are transferred instead of a single gene, or sometimes thousands of nucleotides are mutated instead of a single nucleotide. Therefore, transgenic breeding programs continued to evolve the end of the 20th century (1995) to overcome such problems. The 21st century is regarded as

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The image shows a workflow for accessing a full-text PDF. On the left, a search result for the article "Genome Editing Tools in Plants" is shown. The article title and authors (Mohanta, TK; Bashir, T; Bae, H) are highlighted in yellow. A purple box highlights the "View PDF" button. An arrow points from this button to the right, where the full article page is displayed. The article page includes the journal logo (genes), the MDPI logo, the title "Genome Editing Tools in Plants", the authors' names, and their affiliations. On the right side of the article page, there is a sidebar with a "My Locker" section containing a "Saved in Locker" button and several action buttons: "Download PDF", "Share PDF", "Export to EndNote", "Push to EndNote account", "Visit journal page", "Get citation", "Manage tags", and "Web of Science record".

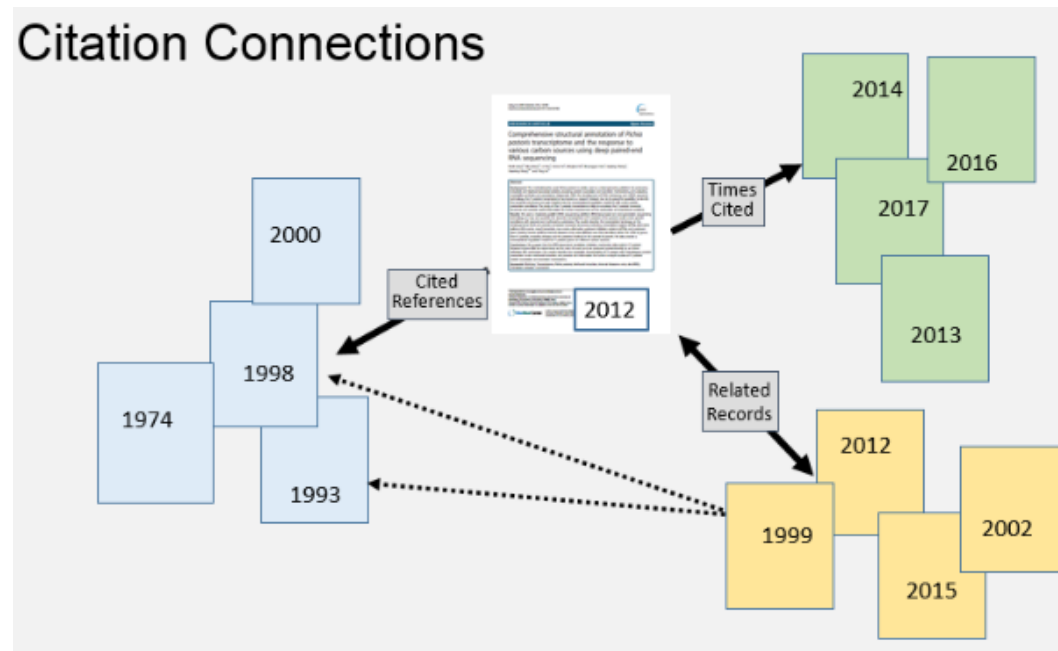
15 **Genome Editing Tools in Plants** 15 Citations
Mohanta, TK; Bashir, T; (...); Bae, H
Dec 2017 | *Genes*
177 References
Genome editing tools have the potential to change the genomic architecture of a genome at precise locations, with desired accuracy. These tools have been efficiently used for trait discovery and for the generation of plants with high crop yields and resistance to biotic and abiotic stresses. Due to complex gei ... [Show more](#)
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Review
Genome Editing Tools in Plants
Tapan Kumar Mohanta ^{1,*}, Tufail Bashir ¹, Abeer Hashem ^{2,3}, Elsayed Fathi Abd_Allah ⁴
and Hanhong Bae ^{1,*}
¹ Department of Biotechnology, Yeungnam University, Gyeongsan 38541, Korea; tufail.arab@gmail.com
² Botany and Microbiology Department, College of Science, King Saud University, Riyadh 11451, Saudi Arabia; habeer@ksu.edu.sa
³ Mycology and Plant Disease Survey Department, Plant Pathology Research Institute, Agriculture Research Center, Giza 12619, Egypt
⁴ Plant Production Department, College of Food and Agriculture Science, King Saud University, Riyadh 11451, Saudi Arabia; eabdallah@ksu.edu.sa
* Correspondence: nostoc.tapan@gmail.com (T.K.M.); hanhongbae@ynu.ac.kr (H.B.)
Received: 16 November 2017; Accepted: 15 December 2017; Published: 19 December 2017

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- Cites References – the research that a paper cites (all cited reference are captured, regardless whether they are part of the index or not)
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Discover Related Records that have a topical association to CRISPR research papers

View a list of records that cite at least one document cited by the parent record identified. Related Records are ranked according to the number of references they share with the parent record. The assumption behind Related Records searching is that articles that cite the same works have a subject relationship, regardless of whether their titles, abstracts, or keywords contain the same terms. The more cited references two articles share, the closer the subject relationship.

Out of the 15,344 Related Records: 7,307 contain the keywords "Clustered Regularly Interspaced Short Palindromic Repeat" or CRISPR while 8,307 records do not!

Multiplex Genome Engineering Using CRISPR/Cas Systems

By: Cong, L (Cong, Le)^{1, 2, 3}; Ran, FA (Ran, F. Ann)^{1, 2, 5}; Cox, D (Cox, David)^{1, 2, 4}; Lin, SL (Lin, Shuailiang)^{1, 2, 6}; Barretto, R (Barretto, Robert)⁷; Habib, N (Habib, Naomi)^{1, 2}; Hsu, PD (Hsu, Patrick D.)^{1, 2, 5}; Wu, XB (Wu, Xuebing)^{8, 9}; Jiang, WY (Jiang, Wenyang)¹⁰; Marraffini, LA (Marraffini, Luciano A.)¹⁰; Zhang, F (Zhang, Feng)^{1, 2...Less}
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SCIENCE
Volume: 339 Issue: 6121 Pages: 819-823
DOI: 10.1126/science.1231143
Published: FEB 15 2013
Document Type: Article

Abstract
Functional elucidation of causal genetic variants and elements requires precise genome editing technologies. The type II prokaryotic CRISPR (clustered regularly interspaced short palindromic repeats)/Cas adaptive immune system has been shown to facilitate RNA-guided site-specific DNA cleavage. We engineered two different type II CRISPR/Cas systems and demonstrate that Cas9 nucleases can be directed by short RNAs to induce precise cleavage at endogenous genomic loci in human and mouse cells. Cas9 can also be converted into a nicking enzyme to facilitate homology-directed repair with minimal mutagenic activity. Lastly, multiple guide sequences can be encoded into a single CRISPR array to enable simultaneous editing of several sites within the mammalian genome, demonstrating easy

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- 2021 6
- 2020 1,693
- 2019 1,886

1 Genome engineering using the CRISPR-Cas9 system
Ran, FA; Hsu, PD; (...); Zhang, F
Nov 2013 | Nature Protocols
4,101 Citations
63 References (19 shared)
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2 Genome Engineering Using CRISPR-Cas9 System
Cong, L and Zhang, F
2015 | Chromosomal Mutagenesis, Second Edition
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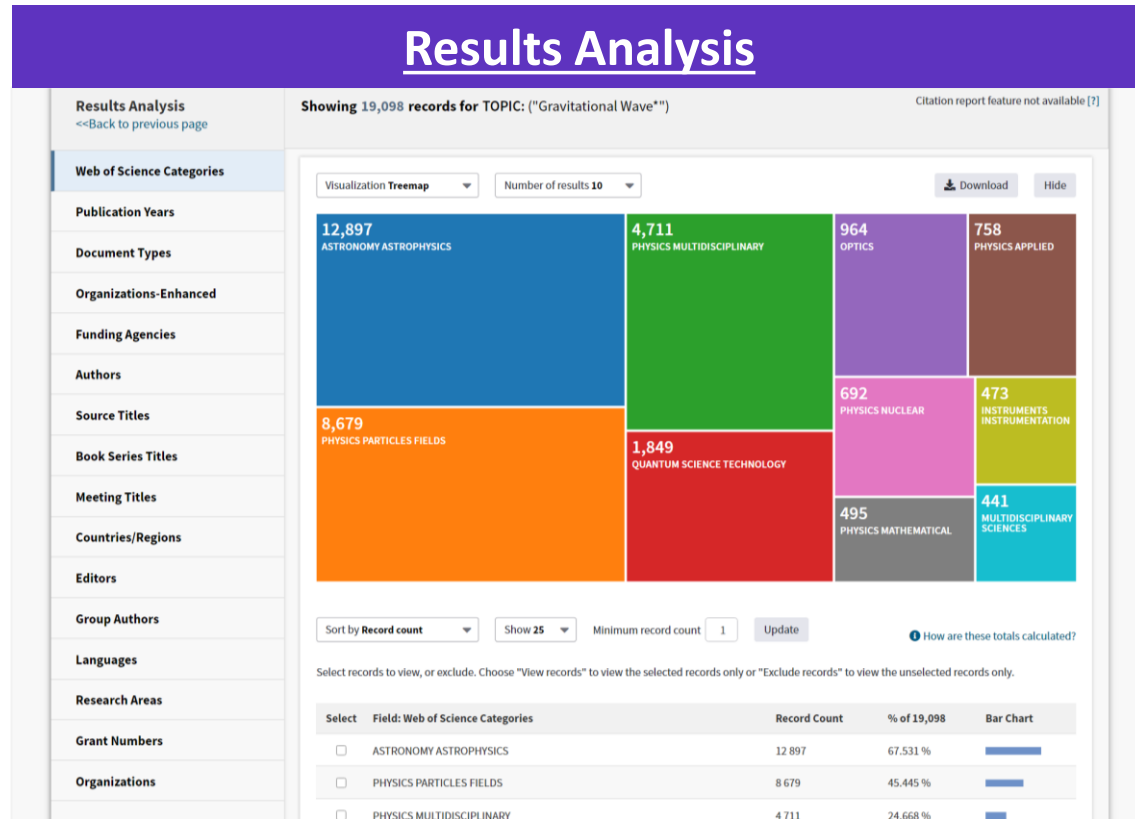
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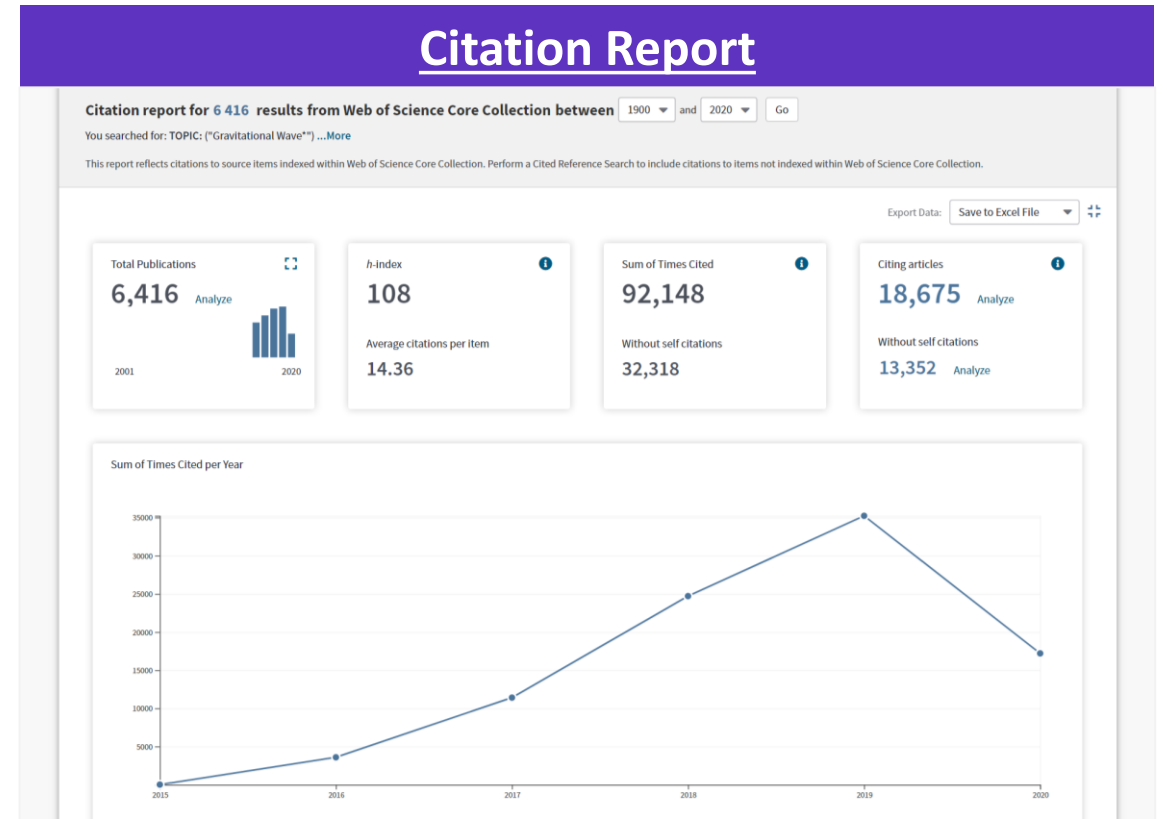
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1 Genome Editing for CNS Disorders

★ Duarte, E and Deglon, N
Oct 22 2020 | [Frontiers In Neuroscience](#) 178 References

Central nervous system (CNS) disorders have a social and economic burden on modern societies, and the development of effective therapies is urgently required. Gene editing may prevent or cure a disease by inducing genetic changes at endogenous loci. Genome editing includes not only the insertion, ... [Show more](#)

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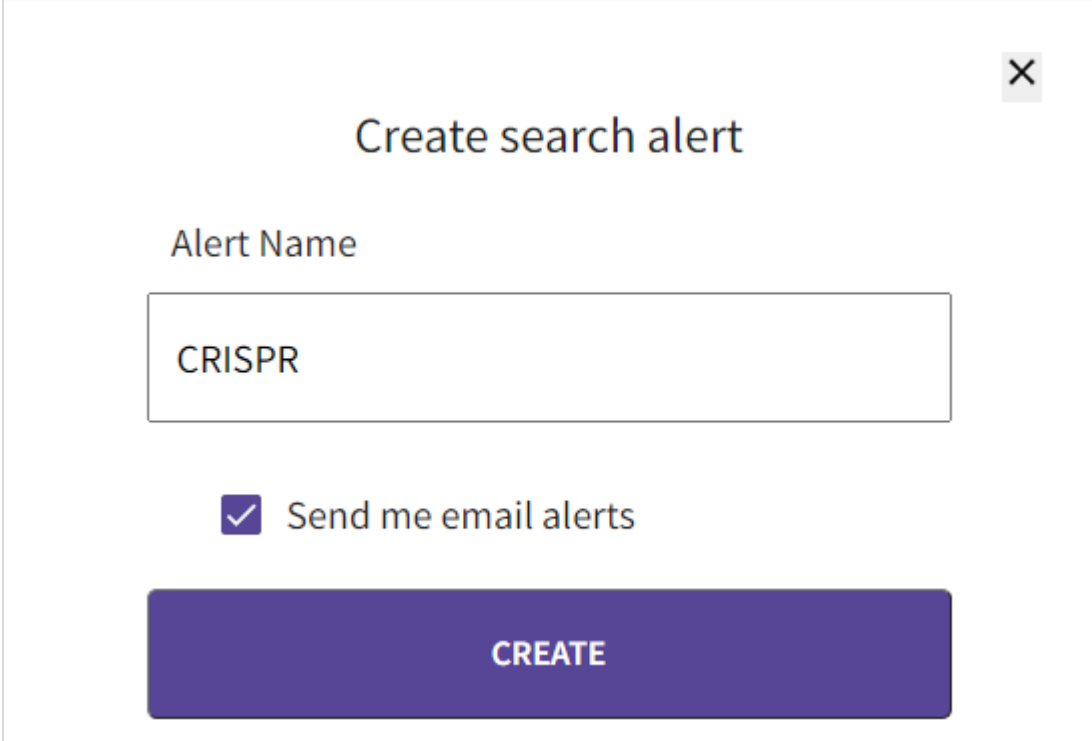
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<input type="checkbox"/> REVIEW (1,957)	<input type="checkbox"/> DATA STUDY (325)	<input type="checkbox"/> UNSPECIFIED (33)	<input type="checkbox"/> REFERENCE MATERIAL (1)
<input type="checkbox"/> MEETING (1,150)	<input type="checkbox"/> PATENT (292)	<input type="checkbox"/> CASE REPORT (16)	<input type="checkbox"/> REPORT (1)
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1. Targeted gene deletion in *Brettanomyces bruxellensis* with an expression-free CRISPR-Cas9 system
Associated Data
By: Varela, Cristian; Bartel, Caroline; Onetto, Cristobal; et al.
APPLIED MICROBIOLOGY AND BIOTECHNOLOGY Volume: 104 Issue: 16 Pages: 7105-7115 Published: AUG 2020
Early Access: JUN 2020
Full Text from Publisher View Abstract
2. Design and Experimental Evaluation of a Minimal, Innocuous Water Strategy to Distinguish Near-Identical DNA and RNA Sequences
Associated Data
By: Boonekamp, Francine J.; Dashko, Sofia; Duiker, Donna; et al.
ACS SYNTHETIC BIOLOGY Volume: 9 Issue: 6 Pages: 1361-1375 Publ 2020
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3. Pivotal role of the transcriptional co-activator YAP in trophoblast stemness of the developing human placenta
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Data Citation Index

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Targeted gene deletion in *Brettanomyces bruxellensis* with an expression-free CRISPR-Cas9 system

From Repository: [European Nucleotide Archive](#)
Group Author(s): [The Australian Wine Research Institute](#)

European Nucleotide Archive
Source URL: <https://www.ebi.ac.uk/ena/browser/view/PRJNAG22385>
Viewed Date: 08 Sep 2020
Published: 2020
Document Type: Data study

Citation Network

In Web of Science Core Collection

1
Times Cited

Create Citation Alert

All Times Cited Counts

Abstract

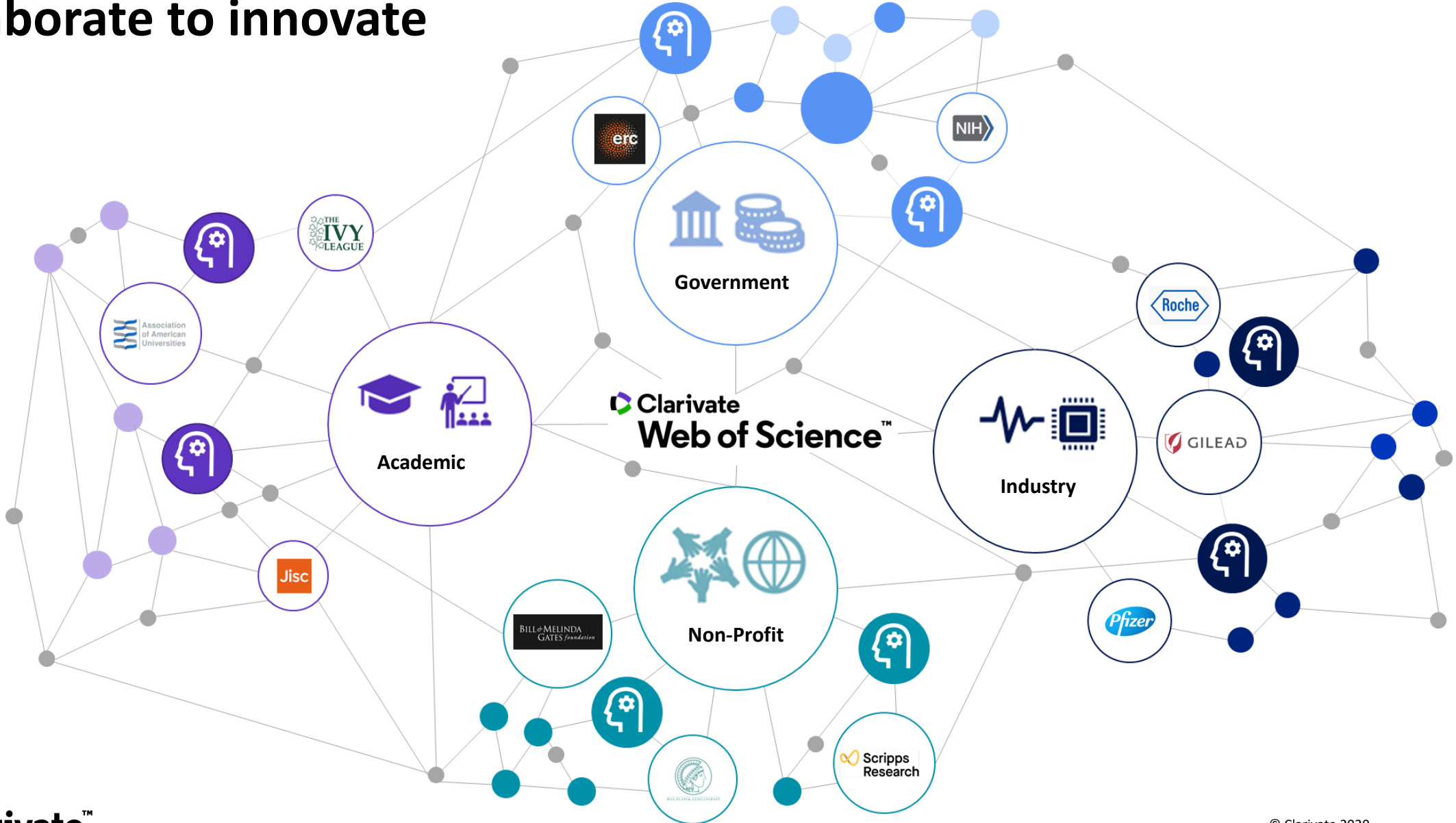
The ability to genetically manipulate microorganisms has been essential for understanding their biology. Targeted genome editing relies on highly efficient homologous recombination, and while this is readily achieved in *Saccharomyces cerevisiae*, most non-conventional yeast species do not display this trait and remain recalcitrant to existing editing methods. CRISPR-based editing can bypass the requirement for high levels of native homologous recombination to be more broadly implemented. While genetic transformation has been reported in *Brettanomyces bruxellensis*, a yeast with broad biotechnological potential and responsible for significant spoilage in the production of fermented beverages, targeted editing approaches have not been reported. Here, we describe an expression-free CRISPR-Cas9 system, in combination with gene transformation cassettes tailored for *B. bruxellensis*, for targeted gene deletion in this species. Deletion efficiency was shown to be dependent on the

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The screenshot shows the Cortellis Clinical Trials Intelligence search results page. At the top, the search bar contains 'CRISPR*' and is highlighted with a purple box. Below the search bar, the results are displayed in a table format. The table has columns for Title, Condition, Patient Segment, Biomarkers, Interventions, and Phase. The search results are sorted by Phase, from lowest to highest. The first result is for 'iHSCs With the Gene Correction of HBB Intervent Subjects With Beta-Thalassemia Mutations'. The second result is for 'Study to Evaluate Safety, Tolerability, Pharmacokinetics, and Pharmacodynamics of NTLA-2001 in Patients With Hereditary Transthyretin Amyloidosis With Polyneuropathy (ATTRv-PN)'. The third result is for 'A Safety and Efficacy Study Evaluating CTX-130 in Subjects With Relapsed or Refractory T or B Cell Malignancies'. The fourth result is for 'A Safety and Efficacy Study Evaluating CTX-130 in Subjects With Relapsed or Refractory Renal Cell Carcinoma'. On the left side, there are filters for Condition, Patient Segment, Biomarkers, Biomarker Type, Biomarker Role, and Drug Pipeline Interventions. The bottom left corner features the Clarivate logo.

Cortellis™ Clinical Trials Intelligence

Advanced search | Structure search | Search history

< Back | Forward > Search Results Related Content | Analyze | Save and Alert | Download

39 results found for index Search for the search term 'CRISPR'

Report Type: **Clinical Trials (39)**

Results Per page: 10 Sort by: Phase Lowest to Highest Order Columns

Results	Title	Condition	Patient Segment	Biomarkers	Interventions	Phase
1	iHSCs With the Gene Correction of HBB Intervent Subjects With Beta-Thalassemia Mutations	Beta thalassemia	Anemia - Subjects with Thalassemia; Anemia - Subjects with specific disease - Subjects with Transfusion Dependent Anemia	Hemoglobin subunit beta	ex vivo HBB-transduced autologous hematopoietic stem cell therapy (beta thalassemia), Allife Medical Science and Technology alone	Phase 0 Clinical
2	Study to Evaluate Safety, Tolerability, Pharmacokinetics, and Pharmacodynamics of NTLA-2001 in Patients With Hereditary Transthyretin Amyloidosis With Polyneuropathy (ATTRv-PN)	Familial amyloid neuropathy	Other neurological disease - Subjects with Treatment Resistant Disease; Other neurological disease - Subjects with specific disease	Body Mass Index; Neurofilament light polypeptide; Transthyretin	transthyretin amyloidosis therapy (CRISPR/Cas9 gene editing/lipid nanoparticles), Regeneron/ Intellia alone	Phase 1 Clinical
3	A Safety and Efficacy Study Evaluating CTX-130 in Subjects With Relapsed or Refractory T or B Cell Malignancies	Diffuse large B-cell lymphoma; T-cell lymphoma	Lymphoma - Subjects with B Cell Non Hodgkin's Lymphoma - Subjects with diffuse large B cell lymphoma; Lymphoma - Subjects with Relapsed/Recurrent Disease; Lymphoma - Subjects with T Cell Non Hodgkin's		CTX-130 alone	Phase 1 Clinical
4	A Safety and Efficacy Study Evaluating CTX-130 in Subjects With Relapsed or Refractory Renal Cell Carcinoma	Metastatic renal cell carcinoma	Renal cell carcinoma - Subjects with Clear Cell Renal Cell Carcinoma; Renal cell carcinoma - Subjects with Metastatic Renal Cell Carcinoma; Renal cell carcinoma - Subjects with Relapsed/Recurrent Disease;		CTX-130 alone; lymphodepleting chemotherapy alone	Phase 1 Clinical

Condition filters:
 Sickle cell anemia (7)
 Beta thalassemia (6)
 Bacterial urinary tract infection (3)
[Show all filters](#)

Filter categories:
 Patient Segment
 Biomarkers
 Biomarker Type
 Biomarker Role
 Drug Pipeline Interventions

Clarivate™

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A clinical study of Aquaporin 1 CRISPR gene therapy for the treatment in patients with glaucoma		◀ Prev	Next ▶
Snapshot	Highlight <input type="checkbox"/> Search Terms & Synonyms	< Previous	Next >
Protocol & Results	SNAPSHOT	Results Available No	
Subjects & Measurements	Title	A clinical study of Aquaporin 1 CRISPR gene therapy for the treatment in patients with glaucoma	
Registry Contacts & Sites	Scientific Title		
Change History	Identifiers		
Sources	Condition	Glaucoma	View Epidemiology in IPD
	Disease Markers		
	Primary Interventions	Aquaporin-1 CRISPR/Cas9 gene therapy (AAV ShH10 serotype, glaucoma) , University of Bristol/UCL Institute of Ophthalmology/University of Cambridge alone	
	Active Controls		
	Phase	Phase Not Applicable	
	Recruitment Status	Planned	
	Reason for Trial Discontinuation		
	Sponsor Only	University of Bristol	
	Collaborator Only	Fight for Sight ; National Eye Research Centre ; Medical Research Council	
	Commercially Relevant	Yes	
	Organization Type	Academic; Government; Non-Government	
	Drug Pipeline Target-based Actions	CRISPR associated endonuclease Cas9 modulator; Aquaporin 1 inhibitor	
	Drug Pipeline Other Actions	Adeno-associated virus based gene therapy	
	Drug Pipeline Technologies	Biological therapeutic; Infusion; Intravenous formulation; Oligonucleotide; Protein recombinant; Virus recombinant	
	Age/Race/Healthy Volunteers		

Cortellis Competitive Intelligence

Cortellis™ All CRISPR* Index Full Text

Advanced search | Structure search | Search history

< Back | Forward > Competitive Landscape Viewer

Timeline & Success Rates Save Download

Indication Action Technology Company

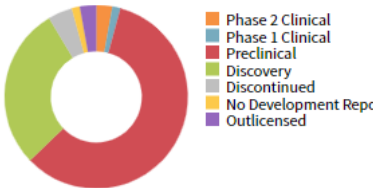
CRISPR associated endonuclease Cas9 modulator Look up

Action Landscape

CRISPR associated endonuclease Cas9 modulator

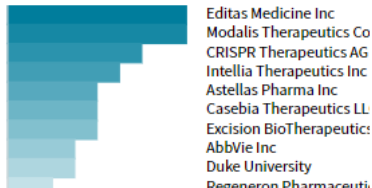
Total Active Drugs 54 Total Companies 47

Status



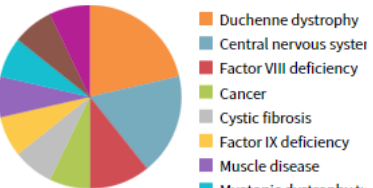
- Phase 2 Clinical
- Phase 1 Clinical
- Preclinical
- Discovery
- Discontinued
- No Development Reperc
- Outlicensed

Companies



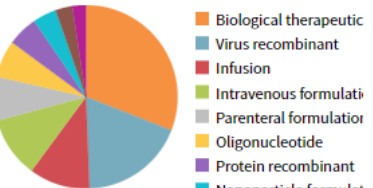
- Editas Medicine Inc
- Modalis Therapeutics Co
- CRISPR Therapeutics AG
- Intellia Therapeutics Inc
- Astellas Pharma Inc
- Casebia Therapeutics LL
- Excision BioTherapeutics
- AbbVie Inc
- Duke University
- Regeneron Pharmaceuti

Indications



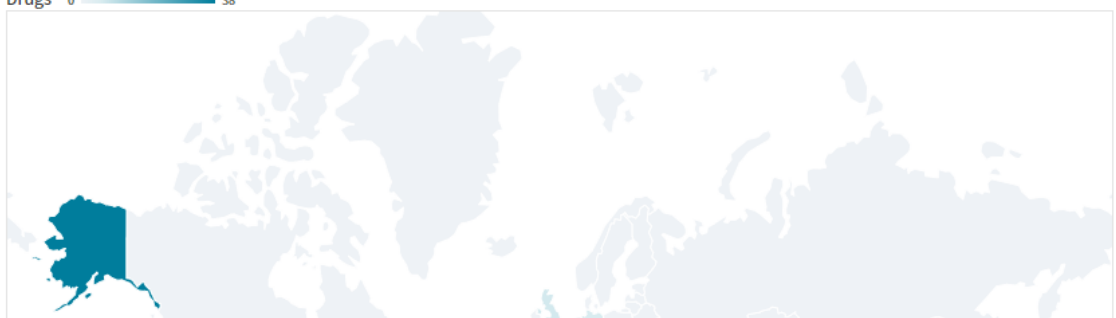
- Duchenne dystrophy
- Central nervous system
- Factor VIII deficiency
- Cancer
- Cystic fibrosis
- Factor IX deficiency
- Muscle disease
- Myotonic dystrophy ty
- Retinitis pigmentosa
- Sickle cell anemia

Technologies



- Biological therapeutic
- Virus recombinant
- Infusion
- Intravenous formulati
- Parenteral formulator
- Oligonucleotide
- Protein recombinant
- Nanoparticle formulat
- Nanoparticle formulat
- Autologous stem cell t

Country/Territory of development



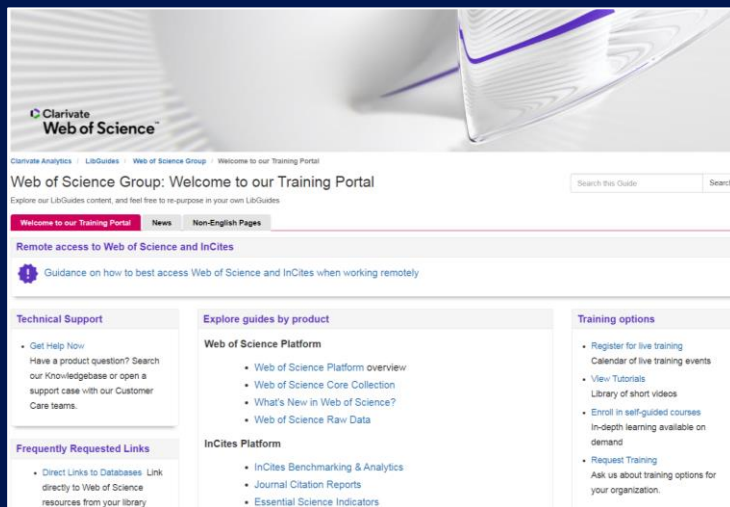
Drugs 0 38

Country/Territory of development

Top five

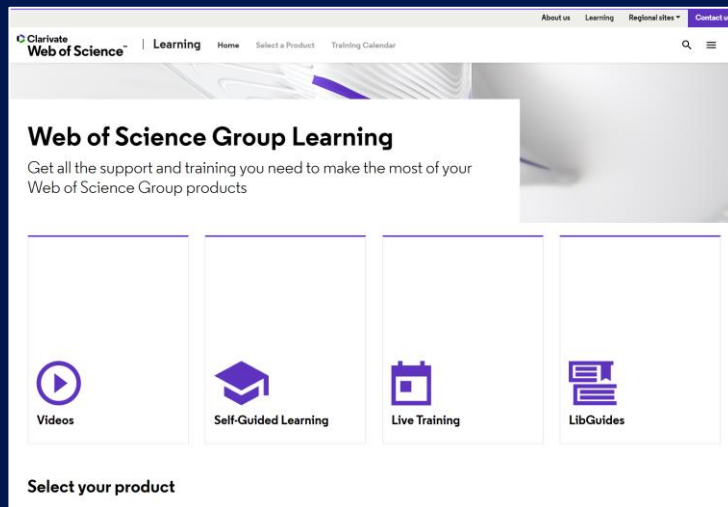
- US (38)
- Japan (8)
- Switzerland (6)
- France (2)

Training resources



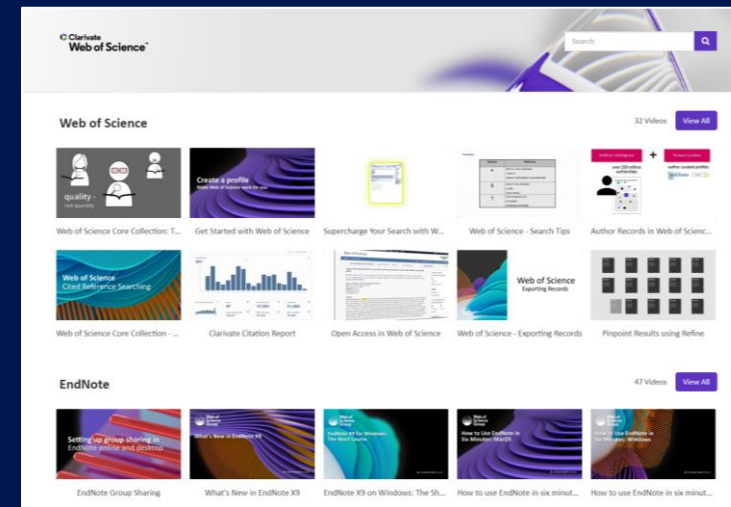
LibGuides

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Vă mulțumesc!

Adriana FILIP

Solutions Consultant

adriana.filip@clarivate.com

www.clarivate.com