## Hasane Ratni

List of Publications by Year in descending order

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HASANE PATNI

#	Article	IF	CITATIONS
1	Risdiplam, the First Approved Small Molecule Splicing Modifier Drug as a Blueprint for Future Transformative Medicines. ACS Medicinal Chemistry Letters, 2021, 12, 874-877.	2.8	38
2	Phenyl bioisosteres in medicinal chemistry: discovery of novel γ-secretase modulators as a potential treatment for Alzheimer's disease. RSC Medicinal Chemistry, 2021, 12, 758-766.	3.9	10
3	Contribution to the Discovery of a Novel Medicine for a Neuromuscular Disease and of other Promising Molecules for the Treatment of Neurodevelopmental and Neurodegenerative Diseases. Chimia, 2021, 75, 614-619.	0.6	Ο
4	SMN protein is required throughout life to prevent spinal muscular atrophy disease progression. Human Molecular Genetics, 2021, 31, 82-96.	2.9	5
5	Discovery of Balovaptan, a Vasopressin 1a Receptor Antagonist for the Treatment of Autism Spectrum Disorder. Journal of Medicinal Chemistry, 2020, 63, 1511-1525.	6.4	35
6	Discovery of RO7185876, a Highly Potent γ-Secretase Modulator (GSM) as a Potential Treatment for Alzheimer's Disease. ACS Medicinal Chemistry Letters, 2020, 11, 1257-1268.	2.8	13
7	Structural basis of a small molecule targeting RNA for a specific splicing correction. Nature Chemical Biology, 2019, 15, 1191-1198.	8.0	89
8	Rewriting the (tran)script: Application to spinal muscular atrophy. Progress in Medicinal Chemistry, 2019, 58, 119-156.	10.4	8
9	A phase 1 healthy male volunteer single escalating dose study of the pharmacokinetics and pharmacodynamics of risdiplam (RG7916, RO7034067), a <i>SMN2</i> splicing modifier. British Journal of Clinical Pharmacology, 2019, 85, 181-193.	2.4	75
10	Risdiplam distributes and increases <scp>SMN</scp> protein in both the central nervous system and peripheral organs. Pharmacology Research and Perspectives, 2018, 6, e00447.	2.4	109
11	Discovery of Risdiplam, a Selective Survival of Motor Neuron-2 ( <i>SMN2</i> ) Gene Splicing Modifier for the Treatment of Spinal Muscular Atrophy (SMA). Journal of Medicinal Chemistry, 2018, 61, 6501-6517.	6.4	324
12	Discovery of a Novel Class of Survival Motor Neuron 2 Splicing Modifiers for the Treatment of Spinal Muscular Atrophy. Journal of Medicinal Chemistry, 2017, 60, 4444-4457.	6.4	26
13	Binding to SMN2 pre-mRNA-protein complex elicits specificity for small molecule splicing modifiers. Nature Communications, 2017, 8, 1476.	12.8	155
14	Pharmacokinetics, pharmacodynamics, and efficacy of a small-molecule <i>SMN2</i> splicing modifier in mouse models of spinal muscular atrophy. Human Molecular Genetics, 2016, 25, 1885-1899.	2.9	28
15	Specific Correction of Alternative Survival Motor Neuron 2 Splicing by Small Molecules: Discovery of a Potential Novel Medicine To Treat Spinal Muscular Atrophy. Journal of Medicinal Chemistry, 2016, 59, 6086-6100.	6.4	83
16	Discovery of Highly Selective Brain-Penetrant Vasopressin 1a Antagonists for the Potential Treatment of Autism via a Chemogenomic and Scaffold Hopping Approach. Journal of Medicinal Chemistry, 2015, 58, 2275-2289.	6.4	43
17	<i>SMN2</i> splicing modifiers improve motor function and longevity in mice with spinal muscular atrophy. Science, 2014, 345, 688-693.	12.6	420