

# 课程大纲

## COURSE SYLLABUS

1.	<b>课程代码/名称</b> Course Code/Title	大分子药物与细胞治疗, 基因治疗 <b>Macromolecule Drugs, Cell and Gene Therapy</b>
2.	<b>课程性质</b> Compulsory/Elective	选修课 Elective
3.	<b>开课单位</b> Offering Dept.	医学院药理学系
4.	<b>课程学分/学时</b> Course Credit/Hours	3/48
5.	<b>授课语言</b> Teaching Language	中英双语 English & Chinese
6.	<b>授课教师</b> Instructor(s)	王鹏 Peng George Wang
7.	<b>开课学期</b> Semester	春季
8.	<b>是否面向本科生开放</b> Open to undergraduates or not	否 No
9.	<b>先修要求</b> Pre-requisites	(如面向本科生开放, 请注明区分内容。 If the course is open to undergraduates, please indicate the difference.) 大学基础生化, 基础化学, 基础有机, 生物学, 药学 College level Biochemistry, Chemistry & Organic Chemistry
10.	<b>教学目标</b> Course Objectives	(如面向本科生开放, 请注明区分内容。 If the course is open to undergraduates, please indicate the difference.)  本课程系统介绍大分子药物/疫苗(包括蛋白药/疫苗, DNA/RNA 药/疫苗, 含糖大分子药/疫苗)与细胞治疗、基因治疗的研究历史, 目前应用和将来的高速发展, 为迎接大分子药物, 细胞治疗与基因治疗在中国的迅速发展和应用打下坚实的基础。 Macromolecule drugs include protein based pharmaceuticals/vaccines, DNA/RNA pharmaceuticals/vaccines and carbohydrate based pharmaceuticals/vaccines. This course will introduce history, current uses and future research/development of macromolecule drugs, cell therapy, gene therapy and will let students gain deep understanding of macromolecule based medicine, and prepare them for the development and use of this fast growing field of pharmaceuticals/vaccines in China.
11.	<b>教学方法</b> Teaching Methods	(如面向本科生开放, 请注明区分内容。 If the course is open to undergraduates, please indicate the difference.)  本课程结合了授课教师大量的实际研究资料及对一线科研文章讨论与学生报告来使学生深刻的学习大分子药物, 细胞治疗与基因治疗。通过结合课堂提问、随堂作业和文献讨论及论文写作等方式来实现实时监测教学质量和及时得到学生反馈。 This course combines a large number of lecturer's practical research materials, discussion of front-line scientific research articles and student reports to enable students to deep understanding of new drug development process, cell and gene therapy. Through the combination of classroom questioning, classroom work, literature discussion and paper writing to achieve real-time monitoring of teaching quality and timely feedback from students.
12.	<b>教学内容</b> Course Contents	(如面向本科生开放, 请注明区分内容。 If the course is open to undergraduates, please indicate the difference.)
	<b>Section 1</b>	1. 大分子药物总论 Introduction to macromolecule drugs 1.1 大分子药物概述 Overview of macromolecular drugs

	<p>1.2 生物大分子药物及其研究现状 Biological macromolecular drugs and their research status.</p> <p>1.3 生物大分子药物传递系统进展 Research progress of macromolecular drug delivery system</p>
<b>Section 2</b>	<p>2. 多肽药物 Polypeptide drugs</p> <p>2.1 多肽药物简介 Introduction of polypeptide drugs</p> <p>2.2 如何对多肽药物进行合理设计 How to design polypeptide drugs rationally</p> <p>2.3 多肽药物的剂型 Dosage forms of polypeptide drugs</p> <p>2.4 多肽药物的市场 The market for polypeptide drugs</p>
<b>Section 3</b>	<p>3. 蛋白药物的选取和研发 Research and development of protein based drugs</p> <p>3.1 蛋白类药物的认识 Knowledge of protein drugs</p> <p>3.2 蛋白药物特点 Characteristics of polypeptide and protein drugs</p> <p>3.3 蛋白类药物的开发技术 Development technology of protein drugs</p>
<b>Section 4</b>	<p>4. 蛋白药物的生产和质量控制 Production and quality control of protein drugs</p> <p>4.1 蛋白药物一般生产工艺 General production technology of protein drugs</p> <p>4.2 蛋白药物的质控要点 Quality control points of protein drugs</p>
<b>Section 5</b>	<p>5. 蛋白药物的药动药代与安全性 Toxicology and ADME of protein drugs</p> <p>5.1 蛋白药物体内外不稳定性 instability of protein drugs in vivo and in vitro</p> <p>5.2 蛋白药物的吸收特征 Absorption characteristics of protein drugs</p> <p>5.3 蛋白药物的给药方法 Administration methods of protein drugs</p> <p>5.4 蛋白药物的代谢与安全 Metabolism and safety of protein drugs</p>
<b>Section 6</b>	<p>6. 最成功的几种蛋白药物 Several most successful protein drugs</p> <p>6.1 PD-1/PD-L1 抗体 PD-1/PD-L1 antibodies——opdivo, keytruda and tecentriq</p> <p>6.2 TNF 抗体 TNF antibody——Humira</p>
<b>Section 7</b>	<p>7. 抗体药物的选取与研发 Research and development of antibody drugs</p> <p>7.1 抗体药物靶点蛋白介绍 Introduction of antibody drug target protein</p> <p>7.2 药物靶点筛选与验证 Screening and verification of drug targets</p> <p>7.3 分子序列确认与制备 confirmation and preparation of molecular sequence</p>
<b>Section 8</b>	<p>8. 抗体药物的生产与质量控制 Production and quality control of antibody drugs</p>

	<p>8.1 抗原抗体的制备与筛选 Preparation and screening of antigen and antibody</p> <p>8.2 抗体功能验证 Verification of antibody function</p> <p>8.3 临床前研究与临床研究 Pre-clinical research and clinical research</p>
<b>Section 9</b>	<p>9. DNA 疫苗 DNA vaccines</p> <p>9.1 DNA 疫苗的定义 Definition of DNA vaccine</p> <p>9.2 DNA 疫苗的设计 Design of DNA vaccine</p> <p>9.3 DNA 疫苗的作用机理 Mechanism of DNA vaccine</p> <p>9.4 DNA 疫苗的优点 Advantages and disadvantages of DNA vaccine</p> <p>9.5 DNA 疫苗的应用 Application of DNA vaccine</p>
<b>Section 10</b>	<p>10. 小核酸药物 iRNA drugs</p> <p>10.1 寡核苷酸药物 Oligonucleotide drugs</p> <p>10.2 ASO 药物 Antisense oligonucleotides drugs</p> <p>10.3 siRNA 药物 small interfering RNA drug</p> <p>10.4 miRNA 药物 microRNA drug</p>
<b>Section11</b>	<p>11. mRNA 疫苗 mRNA vaccines</p> <p>11.1 mRNA 疫苗的原理 Mechanism of mRNA vaccine</p> <p>11.2 mRNA 疫苗的设计 Design of mRNA vaccine</p> <p>11.3 mRNA 疫苗的优势 Advantages of mRNA vaccine</p> <p>11.4 mRNA 疫苗的关键技术 Key technologies of mRNA vaccine</p>
<b>Section12</b>	<p>12. mRNA 药物 mRNA drugs</p> <p>12.1 mRNA 药物发展史 History of mRNA drugs</p> <p>12.2 mRNA 药物的开发 Development of mRNA drugs</p> <p>12.3 mRNA 药物市场 Market of mRNA drugs</p>
<b>Section13</b>	<p>13. 含糖药物总论 Introduction to carbohydrate containing pharmaceuticals</p> <p>13.1 糖类药物的概念 The concept of carbohydrate drugs</p> <p>13.2 糖类药物的特征 Characteristics of carbohydrate drugs</p> <p>13.3 糖类药物的来源 Source of carbohydrate drugs</p> <p>13.4 糖类药物的分类</p>

	<p>Classification of carbohydrate drugs</p> <p>13.5 糖类药物的发展现状 The development status of carbohydrate drugs</p>
<b>Section14</b>	<p>14. 微生物多糖疫苗 Microbial polysaccharides vaccines</p> <p>14.1 微生物多糖疫苗的研究进展 Research progress of microbial polysaccharide vaccines</p> <p>14.2 细菌荚膜多糖结合疫苗 Bacterial capsular polysaccharide conjugate vaccine</p> <p>14.3 寄生虫多糖疫苗 Parasitic polysaccharide vaccine</p> <p>14.4 HIV 多糖疫苗 HIV polysaccharide vaccine</p>
<b>Section15</b>	<p>15. 糖蛋白与糖脂疫苗 Glycoprotein and glycolipid vaccines</p> <p>15.1 糖蛋白与糖脂疫苗简介 Introduction to glycoprotein and glycolipid vaccines</p> <p>15.2 糖蛋白疫苗 Glycoprotein vaccines</p> <p>15.3 糖脂疫苗 Glycolipid vaccines</p>
<b>Section16</b>	<p>16. 坪山药厂参观 Pingshan Pharmaceutical Factory visit</p>
<b>Section17</b>	<p>17. 细胞治疗概述 Introduction to cell therapy</p> <p>17.1 细胞治疗产品的分类 Classification of cell therapy products</p> <p>17.2 细胞治疗产品的安全风险 Safety risks of cell therapy products</p> <p>17.3 细胞治疗产品非临床研究的一般原则 General principles for non-clinical studies of cell therapy products</p>
<b>Section18</b>	<p>18. 干细胞治疗 Stem cell therapy</p> <p>18.1 间充质干细胞在糖尿病治疗中的应用 Application of mesenchymal stem cells in the treatment of diabetes</p> <p>18.2 干细胞在皮肤修复与再生中的应用 Application of stem cells in skin repair and regeneration</p> <p>18.3 间充质干细胞在动脉疾病中的应用 Application of mesenchymal stem cells in arterial diseases</p> <p>18.4 干细胞在心血管病治疗中的应用 Application of stem cells in the treatment of cardiovascular diseases</p>
<b>Section19</b>	<p>19. 免疫细胞治疗 Cellular immunotherapy</p> <p>19.1 实体瘤的 CAR-T 细胞疗法 CAR-T cell therapy for solid tumors</p> <p>19.2 原发性肝细胞癌的免疫治疗 Immunotherapy for primary hepatocellular carcinoma</p> <p>19.3 免疫治疗的新手段——新生抗原免疫疗法 A new approach to immunotherapy ——neoantigen immunotherapy</p> <p>19.4 个性化新抗原肽疫苗治疗胰腺导管的临床研究 Clinical study on the treatment of pancreatic duct with personalized neoantigenic peptide vaccine</p>

<b>Section20</b>	20. CAR-T 细胞治疗 CAR-T cell therapy 20.1 T 细胞基础理论 T cell basic theory 20.2 $\alpha/\beta$ -T 细胞肿瘤抗原的识别机制 $\alpha/\beta$ -T cell tumor antigen recognition mechanism 20.3 $\alpha/\beta$ -T 细胞过继性细胞免疫治疗 $\alpha/\beta$ -T cell adoptive cellular immunotherapy 20.4 $\alpha/\beta$ -T 细胞过继性细胞免疫治疗存在的问题 $\alpha/\beta$ -T cell adoptive cellular immunotherapy has problems
<b>Section21</b>	21. 基因治疗概述 Introduction to gene therapy 21.1 DNA 的结构和基因调控 The structure of DNA and gene regulation 21.2 人类基因组计划及意义 Human Genome Project and Its Significance 21.3 基因编辑技术 Gene editing technology 21.4 基因治疗的临床应用 Clinical application of gene therapy
<b>Section22</b>	22. 靶细胞基因治疗 Target cell gene therapy 22.1 靶细胞基因治疗的概念 The conception of targeting cell gene therapy 22.2 生殖细胞基因治疗 Germ cell gene therapy 22.3 体细胞基因治疗 Somatic gene therapy
<b>Section23</b>	23. 基因治疗的给药途径 Drug administration routes of gene therapy 23.1 ex vivo 途径 ex vivo approach 23.2 in vivo 途径 in vivo approach
<b>Section24</b>	24. 期末报告 Final report
<b>13. 课程考核</b> <b>Course Assessment</b>	
(⊕考核形式 Form of examination; ⊙.分数构成 grading policy; ⊕如面向本科生开放, 请注明区分内容。 If the course is open to undergraduates, please indicate the difference.) 该课程考核方式为考查, 十三等级制: 出勤 Attendance 10 期中考查 Mid-term examination 30 期末考查 Final examination 30 期末报告 Final Presentation 30	
<b>14. 教材及其它参考资料</b> <b>Textbook and Supplementary Readings</b>	
《蛋白质药物—开发与生产》, 那伊、阿凯尔斯主编, 化学工业出版社; 《细胞治疗 CELL THERAPY》, 王明伟主编, 科学出版社; 《基因治疗》, 顾健人主编, 科学出版社。	